Welcome to FDA's Rare Disease Day 2021

Virtual Event

Webcast link:
https://fda.yorkcast.com/webcast/Play/84f0dfdc2c1444679516d9f73d690b351d

In this packet you will find:

- **Agenda**
- **Conference Speaker and Moderator Brief Biographies**

Agenda for FDA Rare Disease Day 2021: Friday, March 5, 2021 (virtual)

9:00-9:10am **Welcome**
Janet Maynard, MD, MHS, Director, Office of Orphan Products Development (OOPD) FDA

9:10-9:15am **Meeting Overview**
Lewis Fermaglich, MD, MHA, Acting Senior Clinical Advisor, OOPD, FDA

9:15-9:30am **Orphan Products Grants Program**
Katherine Needleman, PhD, Director, Clinical Trials and Natural History Grant Program, OOPD, FDA

9:30-9:40am **Opening Remarks**
Amy Abernethy, MD, PhD, Principal Deputy Commissioner of Food and Drugs, Acting Chief Information Officer, FDA

9:40-10:35am **Panel 1: Rare Disease Partnerships, Collaborations, and Scientific Advancements**

Session Goal/Overview:
Provide perspectives on successful partnerships to support rare disease product development. Outline the importance of working with rare disease stakeholders to ensure scientific advancements support the development of rare disease products. Mr. Kroslowitz and Dr. McCormack will provide presentations at the start of the panel followed by a facilitated discussion with all panel members.

Moderator: Susan McCune, MD, Director, Office of Pediatric Therapeutics, FDA

Panelists:

- Robert Kroslowitz, President and CEO of Berlin Heart Inc.
- Frank McCormack, MD, Professor of Medicine; Director, Division of Pulmonary, Critical Care, and Sleep Medicine, University of Cincinnati
- Vasum Peiris, MD, MPH, Chief Medical Officer and Director – Pediatrics and Special Populations, Center for Devices and Radiological Health (CDRH), FDA
- Sally Seymour, MD, Director, Division of Pulmonology, Allergy, and Critical Care (DPACC), Center for Drug Evaluation and Research (CDER), FDA

10:35-10:45am **Break**

10:45-11:45am **Panel 2: Patient Engagement in Rare Disease Product Development**

Session Goal/Overview:
Examine tangible examples of patient engagement in rare disease product development and include a discussion of the importance of natural history studies in rare disease product development. The panelists will provide presentations at the start of the panel followed by a facilitated discussion with the panel members.

Moderator: Robyn Bent, RN, MS, CAPT, U.S. Public Health Service, Director, CDER PFDD Program, FDA

Panelists:

- Wen-Hann Tan, BMBS, Attending Physician, Division of Genetics and Genomics, Associate Professor of Pediatrics, Harvard Medical School
- Amanda Moore, CEO of the Angelman Syndrome Foundation
- Martin Ho, MS, Associate Director of Science for Patient Inputs and Real-World Patient Evidence, Office of Biostatistics and Epidemiology, Center for Biologics Evaluation and Research (CBER), FDA
- Andrea Furia-Helms, MPH, Director, Office of Patient Affairs, Office of Clinical Policy and Programs, FDA

11:45am-12:45pm **Lunch**

12:45-1:00pm **Introductory Remarks for the Afternoon**
Janet Woodcock, MD, Acting Commissioner of FDA

1:00-2:00pm **Panel 3: Strategies to Support Rare Disease Product Development During COVID-19**

Session Goal/Overview:
Discuss strategies to facilitate rare disease product development during the COVID-19 pandemic. Ms. Sher and Dr. Johnson will provide presentations at the start of the panel followed by a facilitated discussion with all panel members.

Moderator: M. Khair ElZarrad, PhD, MPH, Deputy Director, Office of Medical Policy, CDER, FDA

Panelists:

- Rachel Sher, JD, MPH, Vice President, Policy and Regulatory Affairs, National Organization for Rare Disorders (NORD)
- Nicholas E. Johnson, MD, Associate Professor, Vice Chair of Research, Neuromuscular Division Chief, Department of Neurology, Virginia Commonwealth University
- Christine Mueller, DO, Medical Officer, Office of Orphan Products Development, FDA
- Christopher P. Austin, MD, Director, National Center for Advancing Translational Sciences

2:00-2:10pm **Break**

2:10-2:50pm **Panel 4: Discussion with FDA Center Directors Regarding Rare Disease Product Development in the Medical Product Centers**

Session Goal/Overview: Provide Center perspectives on new challenges and opportunities for rare disease product development. The Center Directors will provide remarks at the start of the panel followed by a facilitated discussion with all panel members.

Moderator: Erika Torjusen, MD, MHS, Director, Pediatric Device Consortia and Rare Pediatric Disease and Humanitarian Use Device Designation Programs, OOPD, FDA

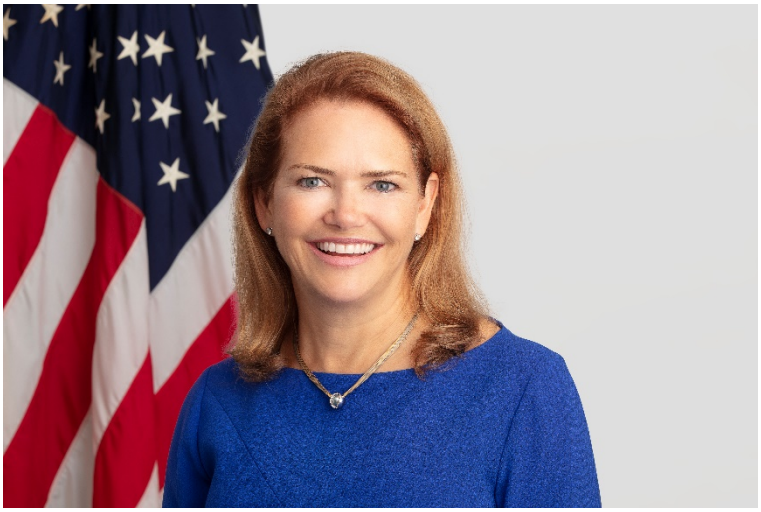
Panelists:

- Peter Marks, MD, PhD, Director, CBER, FDA
- Jeffrey Shuren, MD, JD, Director, CDRH, FDA
- Patrizia Cavazzoni, MD, Acting Director, CDER, FDA

2:50-3:50pm **Open Public Comment Period**
Moderator: Catherine Park, Project Management Officer, OOPD

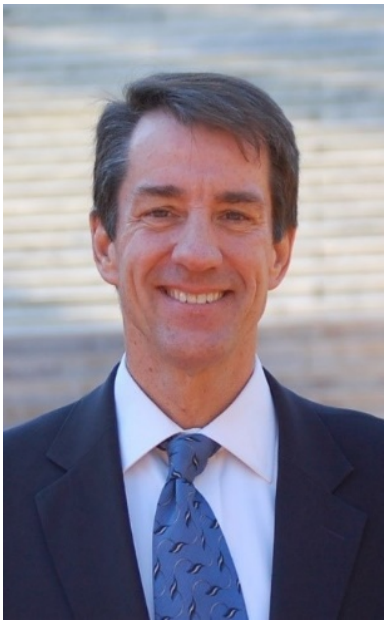
3:50-4:00pm **Closing Remarks**
Janet Maynard, MD, MHS, Director, OOPD, FDA

FDA's Rare Disease Day 2021 Speakers



Amy Abernethy, MD, PhD, Principal Deputy Commissioner of Food and Drugs, Acting Chief Information Officer, FDA

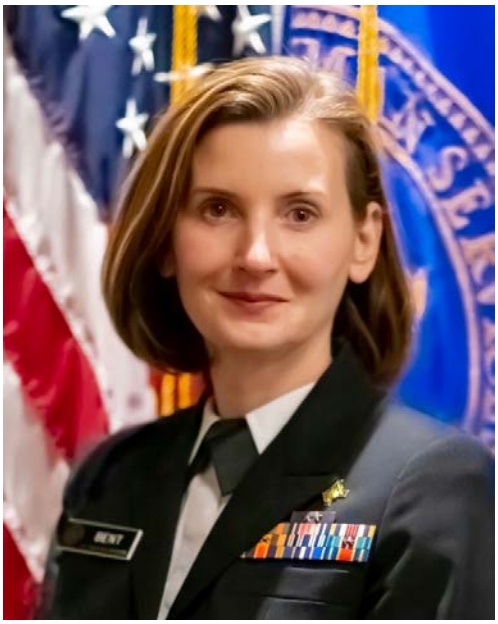
Amy P. Abernethy, M.D., Ph.D. is an oncologist and internationally recognized clinical data expert and clinical researcher. As the Principal Deputy Commissioner of Food and Drugs, Dr. Abernethy helps oversee FDA's day-to-day functioning and directs special and high-priority cross-cutting initiatives that impact the regulation of drugs, medical devices, tobacco and food. As acting Chief Information Officer, she oversees FDA's data and technical vision, and its execution. She has held multiple executive roles at Flatiron Health and was professor of medicine at Duke University School of Medicine, where she ran the Center for Learning Health Care and the Duke Cancer Care Research Program. Dr. Abernethy received her M.D. at Duke University, where she did her internal medicine residency, served as chief resident, and completed her hematology/oncology fellowship. She received her Ph.D. from Flinders University, her B.A. from the University of Pennsylvania and is boarded in palliative medicine.



Christopher P. Austin, MD, Director, National Center for Advancing Translational Sciences

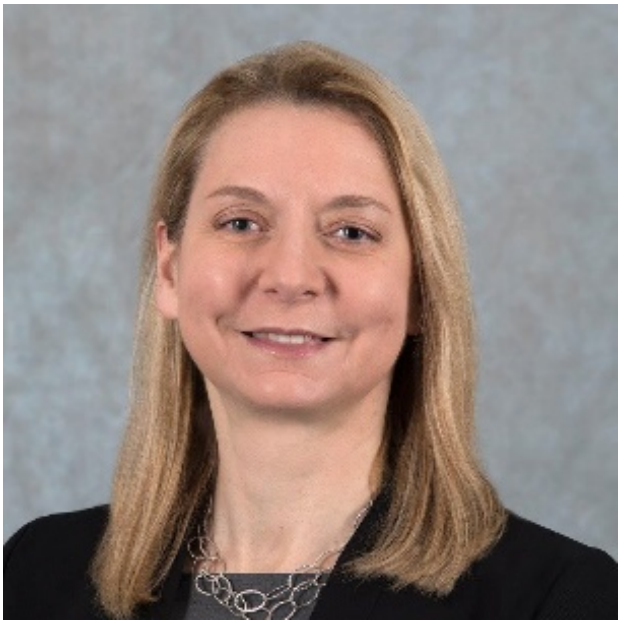
Christopher P. Austin has served as director of the National Center for Advancing Translational Sciences at the National Institutes of Health since 2012. Prior to this role, he was NCATS' scientific director, focusing on translating basic science discoveries into new treatments and technologies to improve the efficiency of therapeutic/diagnostic development. He founded several initiatives, including the NIH Chemical Genomics Center, the Therapeutics for Rare and Neglected Diseases program, and the Toxicology in the 21st Century program. Before joining NIH in 2002, he led genomic-based target discovery, pharmacogenomic, and neuropsychiatric drug-development programs at Merck. From 2016 to 2018, he served as chair of the International Rare Disease Research Consortium

(IRDiRC); Dr. Austin is also a member of National Academy of Medicine. He earned an A.B. from Princeton University, an M.D. from Harvard Medical School, and completed training in internal medicine and neurology at Massachusetts General Hospital.



Robyn Bent, RN, MS, CAPT, U.S. Public Health Service, Director, CDER PFDD Program, FDA

Robyn Bent joined the US FDA in 2019 as the director of the Center for Drug Evaluation and Research (CDER) Patient-Focused Drug Development (PFDD) Initiative, an effort to systematically obtain patient input and facilitate the incorporation of meaningful patient input into drug development and regulatory decision making. The PFDD initiative includes the CDER Standard Core Clinical Outcomes Assessments and Endpoints Pilot Grant Program which provides avenues to advance the use of patient input as an important part of drug development. Prior to joining FDA, Robyn held several positions at the National Institutes of Health. Captain Bent has extensive experience in clinical trial design, conduct, and oversight. Robyn earned her Bachelor of Science in Nursing from The Catholic University of America and her Master of Science degree from the George Washington University.



Patrizia Cavazzoni, MD, Acting Director, CDER, FDA

Dr. Cavazzoni received her medical degree at McGill University and completed a residency in Psychiatry and a fellowship in mood disorders at the University of Ottawa. She subsequently joined the faculty of medicine at the University of Ottawa as an assistant professor, where she was engaged in clinical work, teaching, and research on genetic predictors of mood disorders, authoring numerous peer-reviewed scientific publications. Following this, Dr. Cavazzoni worked in the pharmaceutical industry for several years, and held senior leadership positions in clinical development, regulatory affairs and safety surveillance. Dr. Cavazzoni is certified by the American Board of Neurology

and Psychiatry, a Fellow of the Canadian Royal College of Physician and Surgeons, a member of the Canadian College of Neuropsychopharmacology and recipient of the American College of Psychiatrists' Laughlin Fellowship.

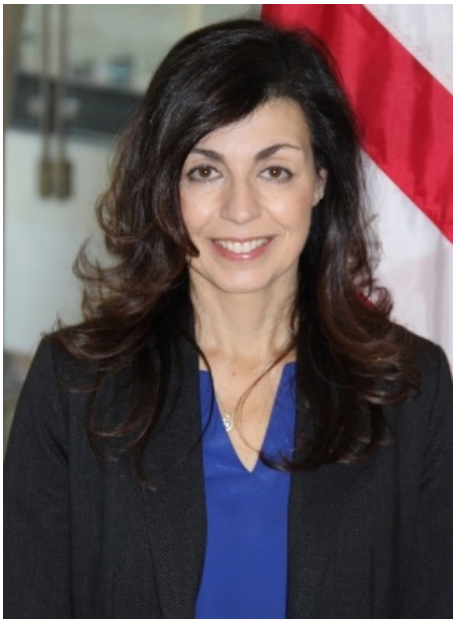
M. Khair ElZarrad, PhD, MPH, Deputy Director, Office of Medical Policy, CDER, FDA

Dr. ElZarrad is the Deputy Director of the Office of Medical Policy (OMP) at FDA's Center for Drug Evaluation and Research (CDER), where he leads the development, coordination, and implementation of medical policy programs and strategic initiatives. Dr. ElZarrad currently leads multiple projects focused on exploring the potential utility of real-world evidence, innovative clinical trial designs, and the integration of technological advances in pharmaceutical development. Dr. ElZarrad is the rapporteur for the International Council for Harmonisation's ongoing work to revise the international Good Clinical Practice Guideline (ICH-E6(R2)). Prior to joining the FDA, he served as Acting Director of the Clinical and Healthcare Research Policy Division with the Office of Science Policy at the National Institutes of Health (NIH). At NIH he worked on policies related to human subject protections; the design, conduct, and oversight of clinical research; and enhancing quality assurance programs at pharmaceutical development and production facilities. He earned a doctoral degree in medical sciences with a focus on cancer metastases from the University of South Alabama, as well as a master's degree in public health from the Johns Hopkins Bloomberg School of Public Health.

Lewis Fermaglich, MD, MHA, Acting Senior Clinical Advisor, OOPD, FDA

Dr. Lewis Fermaglich is a board-certified pediatrician who has been working in The Office of Orphan Products Development (OOPD), first as a Medical Officer and more recently as the Acting Senior Medical Advisor, since September 2019. 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. Additionally, in 2018, he completed an Executive Master of Health Administration at the George Washington University.

Dr. Fermaglich's personal and professional experience, as well as his passion for working with children with rare diseases and their families, has helped to advance OOPD's mission.



Andrea Furia-Helms, MPH, Director, Office of Patient Affairs, Office of Clinical Policy and Programs, FDA

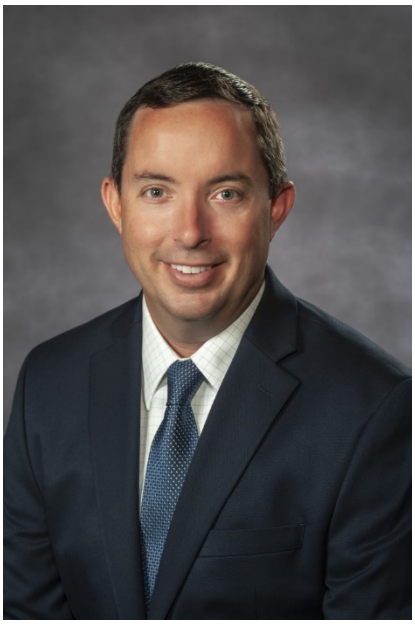
Andrea Furia-Helms is the Director of the Office of Patient Affairs (formerly Patient Affairs Staff) in the Office of Clinical Policy and Programs, Office of the Commissioner. In her role, she collaborates with patient communities, the FDA medical product Centers and other offices to incorporate patient and caregiver perspectives in cross-cutting regulatory meetings. Ms. Furia- Helms spent over ten years in the FDA's Office of Health and Constituent Affairs where she directed the FDA Patient Representative Program and coordinated patient engagement activities for the agency. Prior to FDA, Ms. Furia-Helms was Director of the *Back to Sleep* (now *Safe to Sleep*) campaign, a public-private partnership to educate communities on Sudden Infant Death Syndrome (SIDS), at the National Institutes of Health. She developed SIDS outreach initiatives for African American, American Indian and Latino communities.

Ms. Furia-Helms has a B.A. in psychology from Framingham State University, a B.S. degree in community health education from University of Maryland, and a Master of Public Health from The George Washington University.

Martin Ho, MS, Associate Director of Science for Patient Inputs and Real-World Patient Evidence, Office of Biostatistics and Epidemiology, Center for Biologics Evaluation and Research (CBER), FDA

Martin Ho, MS, is the Associate Director of Science for Patient Inputs and Real-World Evidence at FDA Center for Biologics Evaluation and Research (CBER). He leads a team of multi-disciplinary experts at CBER to evaluate and develop new methods and data sources beyond traditional RCTs to inform regulatory decisions (e.g., clinical outcome assessments, patient preference, real-world evidence, digital health technologies, and site-less clinical trials). Prior to CBER, he worked at FDA Center for Devices and Radiological Health (CDRH) for 10 years. He joined CDRH as a statistical reviewer in 2009 and later became the founding Director of the Quantitative Innovation Program (QIP) at CDRH in 2017. The program was responsible for evaluating novel sources of data to inform regulatory decisions, elevating the appropriate ones to evidence grade through

rigorous and early discussions with the sponsors at the study design stage, and interpreting the study analyses and conclusions from a regulatory perspective. Before joining the FDA, he served as a biostatistician in multiple contract research organizations at different levels and types of responsibilities (e.g., design, conduct, manage, analysis, publications) throughout the clinical trial life cycle for 10 years.



Nicholas E. Johnson, MD, Associated Professor, Vice Chair of Research, Neuromuscular Division Chief, Department of Neurology, Virginia Commonwealth University

Nicholas E. Johnson is an associate professor of Neurology and Human and Molecular Genetics and vice chair of research in Neurology at Virginia Commonwealth University with a focus in inherited neuromuscular disorders. He received his undergraduate degree in molecular and cellular biology and psychology at the University of Arizona. He then obtained his medical degree at the University of Arizona. He completed his neurology residency and combined fellowship in neuromuscular medicine and experimental therapeutics at the University of Rochester.

His laboratory is focused on identifying the pathogenesis of limb girdle muscular dystrophy, myotonic dystrophy, and facioscapulohumeral muscular dystrophy and identifying appropriate clinical endpoints for these conditions. Johnson conducts therapeutic trials in many other inherited nerve and muscle disorders.

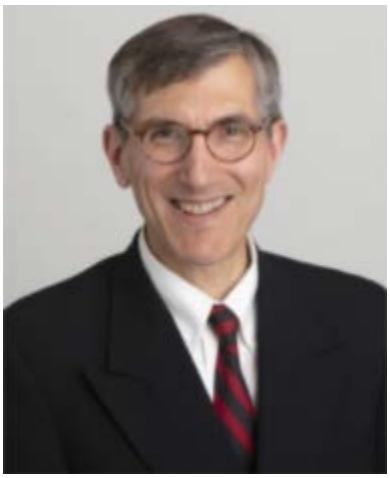
Robert Kroslowitz, President and CEO of Berlin Heart Inc.

Bob Kroslowitz is the President and CEO of Berlin Heart Inc., a company active in the pediatric heart failure space who provides the only FDA approved Ventricular Assist Device System for the pediatric population. Bob joined Berlin Heart in 2004 and was instrumental in the founding and establishment the company's North American Business Unit. He has been responsible for building the business within the marketplace and expanding the company's presence throughout North America.

Bob is passionate about fostering the development of medical devices specifically for the pediatric population and often speaks and serves on panels

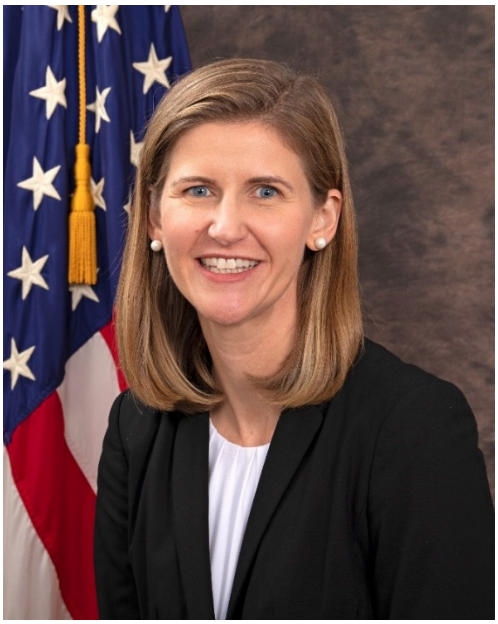
related to this topic. He serves on the Board of Directors for the American Society of Artificial Internal Organs, the Advisory Board for HeartWorks, a non-profit foundation that strives to accelerate a new standard of care for patients with congenital heart disease by developing an innovative culture and an ecosystem of medical experts, institutional collaborators, and activated communities to develop disruptive innovation. Bob is also active FDA's Pediatric Device Consortia program, serving on the Oversight Committees for the Philadelphia Pediatric Medical Device Consortium and the Southwest National Pediatric Device Consortium.

Prior to Joining Berlin Heart Bob held leadership and consulting roles in other medical device companies including Maquet, Jostra, Terumo, and Sorin.



Peter Marks, MD, PhD, Director, CBER, 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 January 2016.



Janet Maynard, MD, MHS, Director, Office of Orphan Products Development (OOPD), FDA

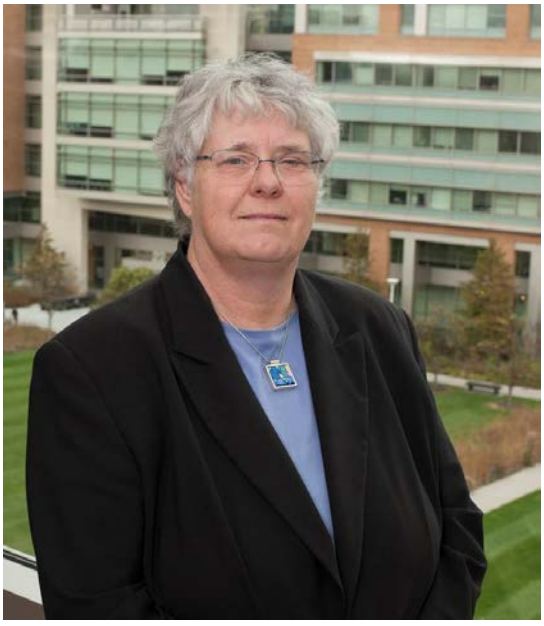
Dr. Janet Maynard, as the Director of the Office of Orphan Products Development (OOPD), oversees legislatively mandated designation and grant programs intended to promote the development of products for rare diseases including, orphan drug, rare pediatric disease, and humanitarian use device designation programs, as well as clinical trial, natural history study, and pediatric device consortia grant programs. Prior to OOPD, Dr. Maynard worked in the Center for Drug Evaluation and Research (CDER), where she was a clinical team leader in the Division of Anesthesia, Analgesia, and Addiction Products (DAAAP). Dr. Maynard has been with FDA since 2011, when she joined FDA's Division of Pulmonary, Allergy, and

Rheumatology Products (DPARP) as a Medical Officer, before becoming a clinical team leader in DPARP.

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

Frank McCormack, MD, Professor of Medicine; Director, Division of Pulmonary, Critical Care, and Sleep Medicine, University of Cincinnati

Dr. McCormack is the J. Gordon and Helen Hughes Taylor Professor of Medicine and Director of the Division of Pulmonary, Critical Care and Sleep Medicine. His research program is focused on clinical trials in the rare lung disease of women, lymphangioleiomyomatosis (LAM), and in the laboratory, on pulmonary innate immunity and the molecular pathogenesis of genetic interstitial lung diseases such as LAM and pulmonary alveolar microlithiasis. He has served on the FDA Pulmonary and Allergy Drug Advisory Council since 2013.

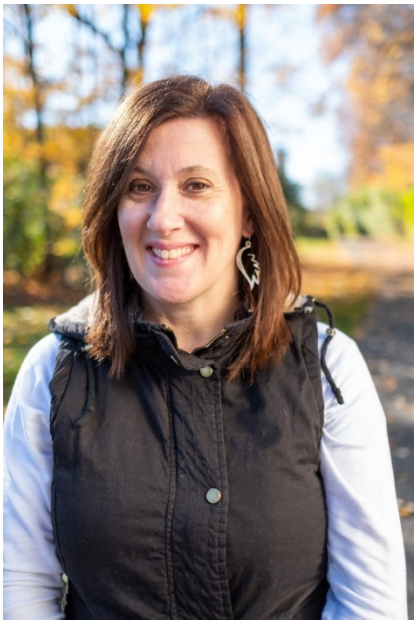


Susan McCune, MD, Director, Office of Pediatric Therapeutics, Office of Clinical Policy and Programs, FDA

Dr. Susan McCune is the Director in the Office of Pediatric Therapeutics (OPT) in the Office of the Commissioner at the Food and Drug Administration (FDA). She joined the Agency in 2003 in the Division of Pediatric Drug Development, Office of Counter-Terrorism and Pediatric Drug Development, in the Center for Drug Development and Research (CDER). She was the Deputy Director in the Office of Translational Sciences in CDER from February, 2010, until January, 2017, when she joined OPT.

Dr. McCune received her medical degree from George Washington University following her undergraduate degree at Harvard University. She completed her internship, residency, chief residency, and neonatal fellowship at

Children's National Medical Center in Washington, D.C. She is Board Certified in Pediatrics and Neonatal/Perinatal Medicine. For 15 years, while practicing academic pediatric and neonatal medicine at Johns Hopkins and Children's National Medical Center, Dr. McCune continued her molecular biology research on adrenergic receptor ontogeny and expression in models of newborn brain injury in the Lab of Developmental Neurobiology, NICHD, NIH. In addition, she has a Master's in Education Technology Leadership from George Washington University and certificates in Public Health from Georgetown and Regulatory Science from USC.



Amanda Moore, CEO of the Angelman Syndrome Foundation

Amanda Moore is the CEO of the Angelman Syndrome Foundation. Before coming to the foundation , she served in executive leadership positions for the YMCA of Greater Indianapolis. She has her Bachelors in Psychology and a Master's degree in Leadership Development. In 2015, Amanda and her husband Adam were blessed with adopting twin boys Jackson and Baden. In 2016, Jackson was diagnosed with Angelman Syndrome and at that time Amanda vowed to take action to do what she could for Jackson and individuals like him. She served on the Angelman Syndrome Board for two years and now is serving as their Chief Executive Officer.

Christine Mueller, DO, Medical Officer, Office of Orphan Products Development, FDA

Christine M. Mueller, D.O. is a Medical Officer in FDA's Office of Orphan Products Development (OOPD) and primarily focuses on the clinical trials and natural history grants programs and works closely with researchers and organizations to advance promising medical products to market approval. She has also worked on orphan drug designations and related issues such as personalized medicine and tissue agnostic therapies. Dr. Mueller joined FDA's Center for Drug Evaluation and Research (CDER) in the Division of Gastroenterology Products in 2008 as a medical reviewer for products to treat inborn errors of metabolism, and then joined OOPD in January of 2010. Dr. Mueller completed her medical education at the Ohio University College of Osteopathic Medicine and completed a residency in Family Medicine in the Cleveland Clinic Health System and a Clinical Genetics fellowship at the University of Pittsburgh Medical Center. Prior to joining FDA, Dr. Mueller was Assistant Clinical Professor in the Department of Family Medicine and Center for Medical Genetics at the University of Pittsburgh Medical Center, and a Clinical Cancer Genetics Research Fellow and Staff Clinician in the Clinical Genetics Branch, Division of Cancer Epidemiology and Genetics at the National Cancer Institute and Program Director in the National Institute of Health's Common Fund in the Office of Strategic Coordination. Dr. Mueller is board certified in Family Medicine and Clinical Genetics.

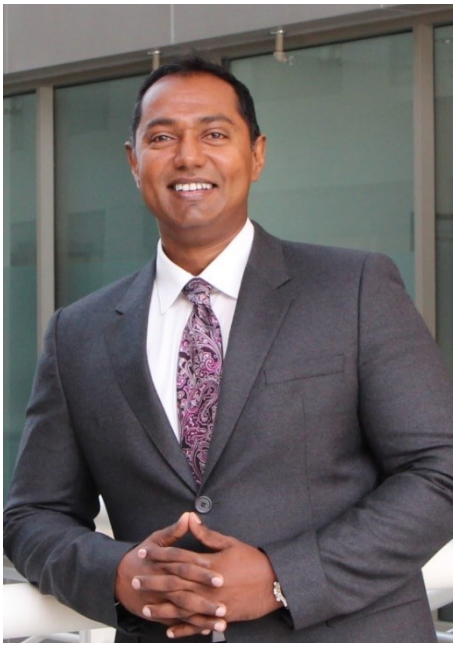
Catherine Park, Project Manager, Office of Orphan Products, FDA

Catherine Park is a project manager of the Rare Pediatric Disease (RPD) designation program, the Humanitarian Use Device (HUD) designation program, and the Pediatric Device Consortia (PDC) at the Food and Drug Administration in the Office of Orphan Products Development (OOPD). She is also a coordinator for FDA's Rare Disease day.

Katherine Needleman, PhD, Director, Clinical Trials and Natural History Grant Program, OOPD, FDA

Katherine Needleman serves as the Director for the Orphan Products Grants Program in the Office of Orphan Products Development (OOPD) at the Food and Drug Administration (FDA). She manages the $17 million OOPD extramural research budget that is used to support grants in the Orphan Products Clinical Trials Grants Program and the Orphan Products Natural History Grants Program. She works closely with project officers, researchers, and organizations to advance promising medical products for rare diseases or conditions to market approval, to increase publications of significant findings in the scientific literature, and to oversee the responsible use of federal funds.

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



Vasum Peiris, MD, MPH, FAAP, FACC, FASE, Chief Medical Officer and Director Pediatrics and Special Populations, Center for Devices and Radiological Health, FDA

As Chief Medical Officer and Director for Pediatrics and Special Populations at the FDA CDRH, Vasum provides strategic vision and executive, clinical and scientific leadership for the Center and Agency. He serves as the Center's senior expert on pediatric clinical science and practice, leading development and implementation of novel strategies, programs and initiatives optimizing innovation, development, assessment, regulation and safe use of medical devices intended for pediatric and special populations. His dynamic leadership engenders relationships and synergy within and among matrixed public and private sector organizations that enhance the ability of the Agency to optimally fulfil its public health mission. Vasum's collaborative, innovative, and cross-cutting work at the FDA has been recognized with multiple awards including the Commissioner's Special Citation and the Commissioner's Award of Excellence.

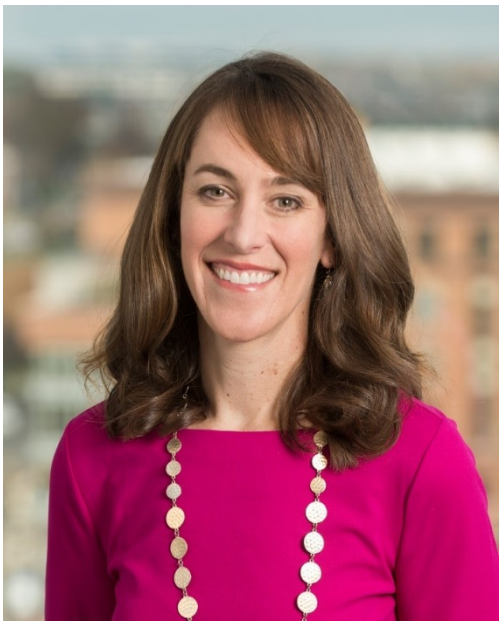
Prior to joining the FDA, Vasum was the Joon Park, M.D. Endowed Chair for Medical Excellence and Chief of Pediatric and Adult Congenital Cardiology at Texas Tech University Health Sciences Center. In addition to leading, expanding and integrating a complex multi-subspecialty clinical service across the health system enterprise, and being inaugural faculty that created the department of public health, Vasum served in multiple additional senior leadership roles for the University and Medical Center.

Vasum is triple Board-certified in Pediatrics, Pediatric Cardiology and Adult Congenital Cardiology by the American Board of Pediatrics and the American Board of Internal Medicine. He has been invited faculty for national and international scientific and professional conferences, an invited visiting professor and lecturer at top universities and an ad-hoc reviewer for premier scientific journals. He is an accomplished author and nationally recognized research scientist.

Vasum earned his undergraduate degree at Yale University and his graduate degree at the Yale School of Medicine, Department of Epidemiology and Public Health. He completed medical school at The University of Vermont College of Medicine, residency at Yale School of Medicine, and fellowship at Harvard Medical School.

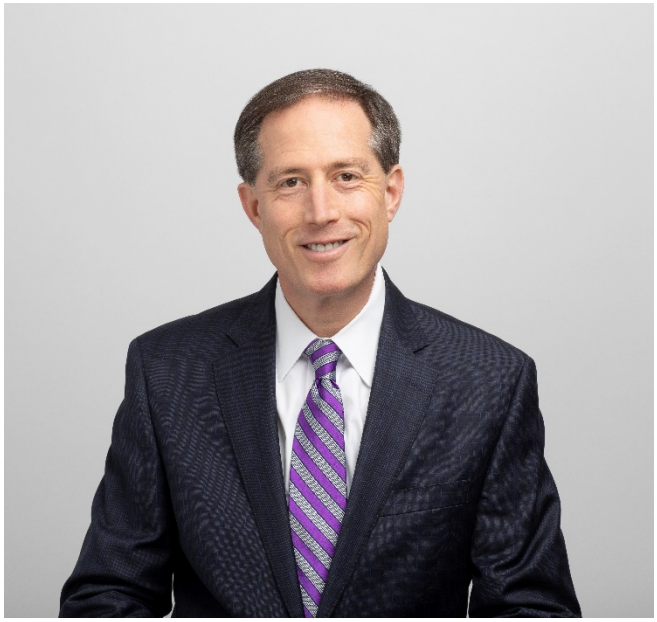
Sally Seymour, MD, Director, Division of Pulmonology, Allergy, and Critical Care (DPACC), Center for Drug Evaluation and Research (CDER), FDA

Sally Seymour received a medical degree from the University of Cincinnati. She trained in internal medicine, pulmonary and critical care medicine at the University of Cincinnati. Since joining FDA in 2003, she has been a medical officer, clinical team leader, and the Deputy Director for Safety for 11 years. She is currently the Director of the Division of Pulmonology, Allergy, and Critical Care at the Center for Drug Evaluation and Research.



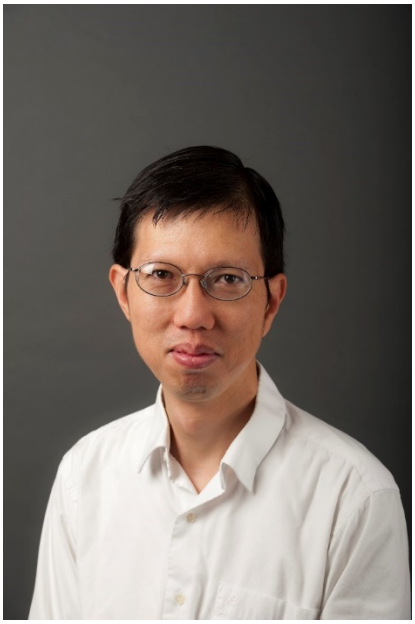
Rachel Sher, JD, MPH, Vice President, Policy and Regulatory Affairs, National Organization for Rare Disorders (NORD)

Rachel Sher Vice President, Policy and Regulatory Affairs Rachel Sher joined the National Organization for Rare Disorders (NORD) in 2019 as Vice President, Policy and Regulatory Affairs, based in the Washington, DC office. Rachel brings nearly 20 years of experience in federal health policy. Prior to joining NORD, Rachel served as Senior Policy Analyst in the Commissioner's Office of Policy at the US Food and Drug Administration (FDA) where she acted as the FDA lead in the 21st Century Cures Act legislative process. For ten years before joining FDA, Rachel served as Senior FDA Counsel for Ranking Member (Formerly Chairman) Henry A. Waxman, the leading author of the 1983 Orphan Drug Act, both on the Democratic Committee Staff for the Energy and Commerce Committee and in Representative Waxman's personal office. Just prior to joining NORD, Rachel served as Deputy General Counsel at the Association for Accessible Medicines. She holds degrees from the University of Florida Levin College of Law (JD), The George Washington University School of Public Health (MPH) and Duke University (BA). Rachel brings this breadth of expertise to leading NORD's strategy for engaging policymakers at the federal and state levels in an effort to effect positive change for the rare disease community.



Jeffrey Shuren, MD, JD, Director, CDRH, FDA

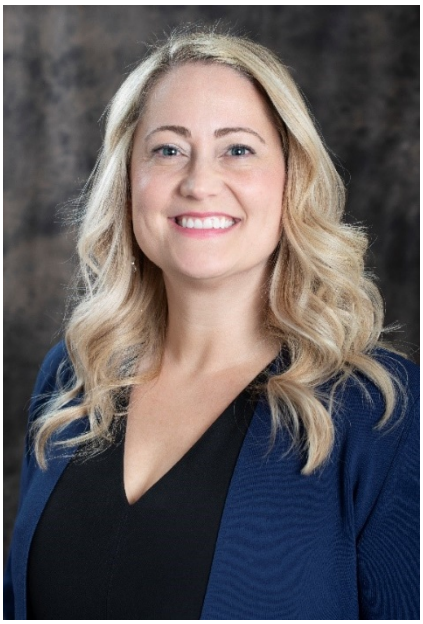
Jeffrey Shuren, MD, JD is the Director of the Center for Devices and Radiological Health (CDRH) at FDA. He previously served as Acting Center Director. Dr. Shuren has held various policy and planning positions within FDA from 1998 to 2009, including Acting Deputy Commissioner for Policy, Planning, and Budget; Associate Commissioner for Policy and Planning; and Special Counsel to the Principal Deputy Commissioner. Dr. Shuren is board certified in Neurology and served as an Assistant Professor of Neurology at the University of Cincinnati. In 1998, Dr. Shuren joined FDA as a Medical Officer in the Office of Policy. In 2000, he served as a detailee on the Senate HELP Committee. In 2001, he became the Director of the Division of Items and Devices in the Coverage and Analysis Group at the Centers for Medicare and Medicaid Services. From 1998 to 2003, he served as a Staff Volunteer in the National Institutes of Health's National Institute of Neurological Disorders and Stroke Cognitive Neuroscience Section supervising and designing clinical studies on human reasoning. Dr. Shuren returned to FDA as the Assistant Commissioner for Policy in 2003 and assumed his current position in September 2009.



Wen-Hann Tan, BMBS, Attending Physician, Division of Genetics and Genomics, Associate Professor of Pediatrics, Harvard Medical School

Wen-Hann Tan is a clinical investigator and geneticist at Boston Children's Hospital with an expertise in the design and execution of natural history studies, patient registries, and clinical trials in rare monogenic disorders. He is currently the PI of a FDA-funded multi-center natural history study of Angelman syndrome, a co-investigator in the Bohring-Opitz syndrome and ASXL-related disorders Registry led by his mentee, Dr. Bianca Russell of University of California, Los Angeles, and a site PI of multiple industry-sponsored clinical trials in Angelman syndrome as well as a first-in-human gene therapy trial in OTC deficiency. He serves on the Scientific Advisory Committee of the Angelman Syndrome Foundation,

and on the advisory board of the Bohring-Opitz Syndrome Foundation and the ASXL Rare Research Endowment Foundation.



Erika Torjusen, MD, MHS, Director, Pediatric Device Consortia and Rare Pediatric Disease and Humanitarian Use Device Designation Programs, OOPD, FDA

Dr. Erika Torjusen is the Director of the Rare Pediatric Disease (RPD) designation program, the Humanitarian Use Device (HUD) designation program, and the Pediatric Device Consortia (PDC) at the Food and Drug Administration in the Office of Orphan Products Development (OOPD). As the Director of these legislatively mandated designation and grant programs, Dr. Torjusen, advances the mission of OOPD, to promote the evaluation and development of products (drugs, biologics, devices, or medical foods) for rare diseases and conditions. In her role, Dr. Torjusen collaborates across the Agency, and with sponsors, stakeholders, and patients on numerous rare disease issues.

Prior to joining OOPD, Dr. Torjusen worked in the Center for Drug Evaluation and Research (CDER), where she was a clinical team leader in the Division of Anesthesia, Analgesia, and Addiction Products (DAAAP). Dr. Torjusen has been with FDA since 2012, when she joined FDA's Division of Pulmonary, Allergy, and Rheumatology Products (DPARP) as a Medical Officer.

At FDA, she has worked with an interdisciplinary team of scientists in evaluating the safety and efficacy of drugs and provided regulatory and scientific guidance to product developers. Dr. Torjusen has served as a key advisor on issues related to individual products, broad policies, and strategic initiatives regarding product development. In addition, she has worked on complex pediatric and rare disease issues, including considerations in cystic fibrosis and pediatric hereditary angioedema. She has served on several Agency working groups and participated in important advisory committee discussions.

Dr. Torjusen received her medical degree from Tufts University School of Medicine and completed a residency in pediatrics at Yale New Haven Children's Hospital. Subsequently, she completed a fellowship in allergy immunology at Johns Hopkins Hospital. During her fellowship training, she completed a Master of Health Science in Epidemiology at the Johns Hopkins Bloomberg School of Public Health.

Janet Woodcock, MD, Acting Commissioner of FDA

As Acting Commissioner, Dr. Woodcock 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. Woodcock began her FDA career in 1986, joining the agency's Center for Biologics Evaluation and Research (CBER) as Director of the Division of Biological Investigational New Drugs, as well as serving as CBER's Acting Deputy Director for a period of time. She later became Director of the Office of Therapeutics Research and Review in CBER, which included the approval of the first biotechnology-based treatments for multiple sclerosis and cystic fibrosis during her tenure.

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. There she led many of the FDA's drug initiatives, including introducing the concept of risk management as a new approach to drug safety; modernizing drug manufacturing and regulation through the Pharmaceutical Quality for the 21st Century Initiative; advancing medical discoveries from the laboratory to consumers more efficiently under the Critical Path Initiative; and launching the Safety First and Safe Use initiatives designed to improve drug safety management within and outside the FDA, respectively.

In 2004, Dr. Woodcock became Deputy Commissioner and Chief Medical Officer in the Office of the Commissioner. Later she took on other executive leadership positions in the Commissioner's Office, including Deputy Commissioner for Operations and Chief Operating Officer.

In 2007, Dr. Woodcock returned as Director of CDER until she was asked to lend her expertise to "Operation Warp Speed" for developing therapeutics during the COVID-19 pandemic, such as evaluating the potential benefits of monoclonal antibody treatments for certain COVID-19

patients. From late 2020, she split her time advising “Operation Warp Speed” on advancing COVID-19 therapeutics while also serving as the Principal Medical Advisor to the Commissioner on key priorities on behalf of the Office of the Commissioner.

Dr. Woodcock holds a Bachelor of Science in chemistry from Bucknell University (Lewisburg, PA), and a Doctor of Medicine from the Feinberg School of Medicine at Northwestern University Medical School (Chicago). She also completed further training and a fellowship in rheumatology, as well as held teaching appointments at the Pennsylvania State University and the University of California in San Francisco. She is board certified in internal medicine.

Dr. Woodcock has been bestowed numerous honors over her distinguished public health career, most notably: 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; and the 2019 Biotechnology Heritage Award from the Biotechnology Innovation Organization and Science History Institute.