

Patient Voice in Rare Disease Medical Product Development

Friday, October 16, 2026 | 1-5pm (eastern)

Speaker Bios

Robyn Bent, MS, RN

Director of Patient-Focused Drug Development (PFDD) Program, CDER, FDA



Robyn Bent, MS, RN, is the director of Patient Focused Drug Development in the Center for Drug Evaluation and Research (CDER) at the U.S. Food and Drug Administration (FDA). Ms. Bent also leads the International Program and is an FDA representative to the International Council for Harmonisation (ICH) Management Committee (MC), currently serving as the MC interim Vice-Chair. Ms. Bent is also the director of the Patient-Focused Drug Development (PFDD) Program in CDER.

Prior to joining the FDA, Ms. Bent held several positions at the National Institutes of Health. She has a background in pediatric oncology nursing, extensive experience in clinical trial design and oversight, and continues to practice as a registered nurse. Ms. Bent earned her Bachelor of Science in Nursing from The Catholic University of America and her Master of Science degree from the George Washington University.

Allyson Berent, DVM

Chief Science Officer, Foundation for Angelman Syndrome Therapeutics



Dr. Allyson Berent is a veterinary internal medicine specialist who serves as the Director of Interventional Endoscopy Services at the largest animal teaching hospital in the world, The Animal Medical Center, in New York City. After graduating from Cornell University College of Veterinary Medicine she completed a residency in Small Animal Internal Medicine at the University of Pennsylvania and fellowships in interventional radiology at University of Pennsylvania and Endourology at Thomas Jefferson University. She served as an Adjunct Assistant Professor in Internal Medicine and Interventional Radiology/ Interventional

Endoscopy at the Veterinary Hospital of the University of Pennsylvania. In 2014 Dr. Berent's daughter was diagnosed with a rare non-degenerative neurogenetic disorder called Angelman syndrome. In October of 2015 she joined the Board of Directors for the Foundation for Angelman Syndrome Therapeutics (FAST), becoming the Chief Science Officer. Dr. Berent helped to spearhead the development and serves as the co-Director of the pre-competitive Angelman Syndrome Biomarker and Outcome Measure

Consortium (A-BOM) in order to bring patient focused outcome measures forward for human clinical.

Through FAST, Dr. Berent collaborates with a team of scientists globally to encourage translational research opportunities, help bring novel cell and gene-targeted therapies forward toward human clinical trials. Through this work Dr. Berent co-founded GeneTx Biotherapeutics in 2017, serving as the Chief Operating Officer, where the team was singularly focused on advancing an antisense oligonucleotide (ASO) therapy through IND enabling studies and a phase 1/2 clinical trial. This company was acquired in 2022 by Ultragenyx Pharmaceuticals. Following this success FAST launched a drug development accelerator called AS2Bio in order to advance various cell and gene therapies to patients living with Angelman syndrome through a venture philanthropy approach including an AAV gene therapy, and non-viral delivered CRISPR gene editor and a hematopoietic ex-vivo stem cell gene replacement program. Dr. Berent serves as the Chief Development Officer of AS2Bio responsible for the clinical and non-clinical activities of the different portfolio programs.

Michelle Campbell, MS, PhD

Associate Director for Stakeholder Engagement and Clinical Outcomes, Office of Neuroscience, Office of New Drugs, CDER, FDA



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. Dr. Campbell joined the FDA in 2014 and previously was a reviewer on the Clinical Outcome Assessments (COA) Staff (now Division of Clinical Outcome Assessment) 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. In Dr. Campbell's current role, she engages in

discussions and reviews considerations in the use of novel endpoints including using digital health technology data to support regulatory decision making. 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.

Amy Comstock Rick, JD
Director, Rare Disease Innovation Hub, FDA



Amy Comstock Rick, JD, is CDER's Associate Director for Rare Disease Strategy and the Director of Strategic Coalitions for FDA's Rare Disease Innovation Hub (the Hub). She serves in a cross-cutting role across FDA's Center for Drug Evaluations and Research (CDER) and Center for Biologics Evaluation and Research (CBER) to facilitate implementation of the Hub. She also works closely with both centers to develop and carry out a rare disease strategic agenda. Ms. Rick, with support from staff in CBER's and CDER's rare disease programs, is the Hub's primary point of engagement for parties external to FDA.

Most recently, Ms. Rick served as Principal Consultant at Leavitt Partners, focusing on health policy matters, with a primary focus on rare disease and medical product development. Before Leavitt Partners, she served as President and Chief Executive Officer of the Food and Drug Law Institute (FDLI), a non-profit organization dedicated to providing an innovative, open, balanced exchange of ideas and viewpoints across the field of food and drug law.

Before joining FDLI, Ms. Rick was Chief Executive Officer of the Parkinson's Action Network. Ms. Rick also served as President of the Coalition for the Advancement of Medical Research and on the Boards of Directors for Research America, the National Health Council, and the American Brain Coalition.

Ms. Rick had previous federal service as a career attorney at the U.S. Department of Education in 1988, focusing primarily on the field of government ethics. She was the Senate-confirmed Director of the U.S. Office of Government Ethics from 2000 to 2003 and Associate Counsel to the President in the White House Counsel's Office from 1998 to 2000. She received a bachelor of arts degree from Bard College and a juris doctor degree from the University of Michigan.

Tom Croce, BSPHarm
Vice President, Global Patient Advocacy & Engagement, Jazz Pharmaceuticals



Tom Croce is vice president for global patient advocacy and engagement at Jazz Pharmaceuticals, the fully integrated biopharmaceutical company with a diverse portfolio of approved and experimental medicines in neuroscience and oncology. Tom and his team are responsible for identification and integration of patient perspectives across the entire development and commercial continuum, while ensuring meaningful representation to patient, caregiver and advocate partners around the world. He is also responsible for building

and implementing the company's social impact strategy. Tom is a member of Jazz' global leadership team as well as its legal and corporate affairs leadership team.

Prior to joining Jazz, Tom was vice president for global patient advocacy at bluebird bio where he contributed to the development and approval of two gene therapies for severe genetic diseases. Tom also educated and activated the advocacy community on the transformative nature of regenerative medicine and furthered THE company's diversity, equity and inclusion initiatives. Earlier, Tom served as vice president, global advocacy relations for Amgen and held related positions at other companies.

A graduate of the Philadelphia College of Pharmacy, University of Sciences, Tom and his family live outside of Boston.

Michael Davis, MD, PhD

Director, Center for Drug Evaluation and Research, FDA



Dr. Michael Davis is the Center Director of the U.S. Food and Drug Administration's Center for Drug Evaluation and Research (CDER). CDER's mission is to ensure that safe, effective, and high-quality drugs are available to the public. To achieve this, CDER regulates the medical products under its jurisdiction throughout their lifecycle - overseeing the development of new and generic drugs, evaluating applications to determine whether drugs should be approved, monitoring the safety of drugs after they are marketed, conducting research to advance regulatory science, and taking enforcement actions to protect the public from harmful products.

Dr. Davis was previously the Deputy Center Director of CDER, serving on the senior leadership team supporting the Center Director by providing executive leadership on strategic initiatives that advanced CDER's mission.

Dr. Davis completed a Medical Scientist Training Program (MD, PhD in pharmacology) at Case Western Reserve University, psychiatric residency training at the Semel Institute for Neuroscience and Human Behavior at UCLA, and a clinical research fellowship at the West Los Angeles VA Mental Illness Research, Education, and Clinical Center. His research interests focused on the development of novel therapeutics for schizophrenia. Following completion of his fellowship in 2014, he was a Staff Psychiatrist at the Michael E. DeBakey VA Medical Center in Houston, TX, and an Assistant Professor in the Baylor College of Medicine Department of Psychiatry and Behavioral Sciences. Dr. Davis is licensed to practice medicine and is board certified in psychiatry.

From 2016-2022, Dr. Davis served in CDER's Office of New Drugs (OND) as a Clinical Team Leader in the Division of Psychiatry, where he led multidisciplinary teams in the review of drugs for the treatment of psychiatric disorders, as well as contributed to regulatory science and policy development. In this role, he received numerous honors

and awards, including the Division of Psychiatry Annual Awards for Individual Supervisor and for Collaboration, and the FDA Outstanding Service Award for leadership in advancing science in psychiatric drug development. Dr. Davis also presented nationally and internationally on regulatory topics such as estimands, digital health technologies, and issues related to development of psychedelic-based therapies. After leaving the agency in 2022, Dr. Davis served from 2022-2025 as Chief Medical Officer for a nonprofit medical research organization developing psychedelic drugs for the treatment of major depressive disorder and post-traumatic stress disorder. In 2025, Dr. Davis returned to the agency.

Mona Elmacken, MD

Lead Physician, Malignant Hematology Branch, Division of Clinical Evaluation Hematology, Office of Clinical Evaluation, Office of Therapeutic Products, CBER, FDA



Dr. Mona Elmacken has been a clinical team leader at the FDA/CBER/OTP/MHB since 2022. She is double board-certified by the American Board of Pediatrics (ABP) in General Pediatrics and Pediatric Hematology/Oncology/Stem Cell Transplantation. Dr. Elmacken received her medical degree from the University of Alexandria, Egypt; and pursued post graduate residency training in General Pediatrics at Mount Sinai School of Medicine in New York, NY. She then, completed a fellowship training program in Pediatric

Hematology/Oncology/Stem Cell Transplantation at New York Medical College, NY. She then joined the faculty at the Department of Pediatrics, divisions of BMT/Immunology at GWU as an Assistant Professor of Pediatrics and a practicing Bone marrow transplantation and Immunology attending. She also served as an attending physician at the Children's Hospital of Michigan before joining the FDA in 2019.

Tracy Gray, MBA, MS, RN

Health Scientist, Patient Engagement Lead, Office of Innovative Development, Office of Strategic Partnership and Technology Innovation, CDRH, FDA



Tracy L. Gray is a licensed Registered Nurse and Health Scientist at FDA's Center for Devices and Radiological Health (CDRH), where she applies her background in public health, nursing workforce development, and patient-centered experience data to support medical device regulation. An effective professional with substantial experience across federal public health agencies, clinical nursing, and the pharmaceutical industry, she brings a multidisciplinary perspective to patient engagement and medical product development.

Before joining FDA, Tracy served as a Supervisory Public Health Analyst at the Health Resources and Services Administration (HRSA), managing multimillion-dollar grant portfolios supporting advanced nursing education and workforce development in rural and medically underserved communities. Her clinical experience in Intensive Care and Telemetry units deepens her understanding of the patient's experience, particularly for those managing serious, multi-system illness. Earlier in her career, she held roles at Merck & Company spanning cardiovascular, infectious diseases, gastroenterology, and vaccines, with work in market research, new drug launches, and communications.

Tracy holds an MS in Clinical Nurse Leadership, an MBA in Business Administration and a BS in Biological Sciences (Microbiology).

Cynthia Grossman, PhD

Director, Division of Patient-Centered Development, Office of Innovative Development, Office of Strategic Partnership and Technology Innovation, CDRH, FDA



Dr. Cynthia Grossman serves as Director of the Division of Patient-Centered Development within the Center for Devices and Radiological Health at the U.S. Food and Drug Administration, where she leads efforts to advance patient-centered approaches across medical product development. As a social and behavioral scientist, Dr. Grossman has spent her career advancing methods to integrate the voices of patients, caregivers, and communities into biomedical research, healthcare delivery, and regulatory science. Her prior work spans government, industry, nonprofits, and academic research, with leadership roles involving real-world evidence, patient-centered outcomes research, behavioral science, and digital health innovation.

Courtney Lias, PhD

Director, Office of In Vitro Diagnostics, Office of Product Evaluation and Quality, CDRH, FDA



Dr. Courtney H. Lias earned her PhD in Biochemistry, Cellular, and Molecular Biology from the Johns Hopkins University School of Medicine. She currently serves as Director of the FDA's Office of In Vitro Diagnostic Devices (OHT7), a position she has held as part of a distinguished career at the Agency spanning more than two decades.

Throughout her tenure, Dr. Lias has been a leading force in advancing the development of novel therapeutic and diagnostic devices across a broad range of clinical areas, including diabetes

management, genetic testing, infectious disease, and drug dosing and monitoring. Under her leadership, OHT7 has authorized groundbreaking companion diagnostic tests in oncology, pioneered new blood-based diagnostics for Alzheimer's disease, and expanded access to in vitro diagnostic testing in home and primary care settings. Her office has also developed innovative regulatory frameworks designed to facilitate and accelerate patient access to emerging medical technologies.

Dr. Lias is also a champion of digital health innovation. She leads forward-looking programs focused on optimizing the integration of digital health and artificial intelligence/machine learning (AI/ML)-based technologies into healthcare delivery, including the Technology-Enabled Meaningful Patient Outcomes (TEMPO) for Digital Health Devices Pilot.

In recognition of her contributions to regulatory science and public health, Dr. Lias received the prestigious Samuel J. Heyman Service to America Medal in Management Excellence in 2017. This honor acknowledged her pivotal role in enabling the efficient development and authorization of the first automated insulin dosing system.

Emily Machiela-Leestma, PhD
Coordinator, HD Benefit-Risk Initiative



Dr. Emily Machiela-Leestma currently coordinates the HD Benefit-Risk Initiative, a pre-competitive collaboration among seven Huntington's disease organizations that is working to build a holistic understanding of how people living with HD and their families view benefit and risk, including patient preference information. She is Board Chair of the Huntington's Disease Youth Organization (HDYO), currently on leave while she serves as the initiative's neutral coordinator. Emily is the founder of Ibex Translational Consulting, holds a PhD in Cell and Molecular Genetics, and comes from an HD family.

Carmen Matos, MA
**Senior Health Scientist, Office of Public Engagement, Office of Public Affairs,
Office of the Commissioner, FDA**



Carmen Matos is a Senior Health Scientist with the U.S. Food and Drug Administration's Office of Public Engagement, where she has dedicated her career to amplifying the patient voice in FDA's regulatory work. Carmen has played a central role in FDA's Patient Listening Session Program, facilitating direct dialogue between patient communities and FDA review divisions across CBER, CDER, and CDRH, and helping to ensure that patient experiences and perspectives are meaningfully incorporated into the regulatory process. She has worked closely with patient organizations, FDA centers, and international regulatory partners

including the European Medicines Agency and Health Canada to advance best practices in patient engagement.

Carmen holds a Master of Arts in Nutritional and Metabolic Biology from Columbia University Medical Center and a Bachelor of Science in Biochemistry and Molecular Biology from the University of Maryland Baltimore County.

Jenn McNary
Principal, Co-Founder, Canary Advisors



Jenn McNary is a trusted voice in the rare disease community, as a mother, public speaker and fierce advocate. Her work in the rare disease space as a thought leader earned her the Ryan's Quest Ryan's Hero award in 2013, and the prestigious 2017 Meyer-Whalley instrument of change award. Formerly as the director of outreach and advocacy at a Massachusetts based non-profit foundation, she was responsible for the organization of the largest FDA advisory committee hearing in history, with over 1000 Duchenne advocates, families, clinicians and researchers in attendance. Jenn has unique experience in the

drug development field, as a parent of children enrolled in clinical trials, an advocate engaging with the regulators and as a patient engagement professional in the life sciences.

Currently, Jenn is the Co-Founder and Principal of Canary Advisors, a boutique patient advocacy consulting firm with a focus on regulatory and access patient engagement. Her other activities include serving as the Founder of One Rare, a non-profit formed to meet the needs of young adults with rare and chronic conditions and raising her children in Massachusetts.

Katie Meyer, MPP**Vice President of Policy Advocacy, and US Market Access, Novocure**

Katie Meyer is the Vice President of Policy, Advocacy and US Market Access at Novocure, where she leads the company's government affairs, public policy, charitable giving, US market access and strategic alliances efforts. She joined Novocure in 2021 and works closely with internal and external stakeholders to advance policies that expand patient access to Novocure's innovative cancer therapy.

Prior to joining Novocure, Katie built her career over nearly 15 years in healthcare policy and government affairs. She previously served as deputy health policy director at the Senate Finance Committee, and as a senior policy advisor to the House Majority Leader. During her time on Capitol Hill, she contributed to the passage of landmark legislation addressing the opioid epidemic, expanding healthcare access for veterans, and creating incentives for new cures and treatments. In 2025, Katie was recognized as a 40 Under 40 in Cancer honoree.

Katie lives in the Washington, DC metropolitan area with her husband, Jason, and their two children.

Karim Mikhail, BPharm, MSc**Director, Center for Drug Evaluation and Research, FDA**

Karim Mikhail serves as the FDA's Director of the Center for Biologics Evaluation and Research (CBER). He joined the FDA in 2025 as a Senior Advisor in the Office of the Commissioner where he led U.S. competitiveness reforms, spanning multiple disciplines from accelerating early clinical development to on-shoring of biopharmaceutical manufacturing. He brings over two decades of leadership experience in leading both large pharmaceutical companies and small biotechnology startups across therapy areas including cardio-metabolism, respiratory, immunology, neuroscience, oncology, women's health, and anti-infectives.

Mr. Mikhail has lived and worked across eight countries on three continents, with trilingual fluency. He has an unparalleled international perspective and deep understanding of the drug development ecosystem from multiple perspectives—regulatory, commercial, and scientific. His clinical development experience spans a range of therapeutic areas, providing deep understanding of the drug development lifecycle from bench to bedside to market.

Mr. Mikhail holds a Master of Science in Biopharmaceutical Management from École supérieure de commerce de Paris (ESCP Business School) and a Bachelor of Pharmacy &

Pharmaceutical Sciences from Cairo University. His Six Sigma Executive Belt certification reflects his commitment to operational excellence. Mr. Mikhail is a distinguished thought leader in pharmaceutical innovation and entrepreneurial business strategy.

Terry Pirovolakis
Founder & CEO, Elpida Therapeutics/CureSPG50



Terry Pirovolakis, Founder and CEO of Elpida Therapeutics and co-founder of CureSPG50 alongside his wife Georgia, began his rare disease journey after their son Michael was diagnosed with SPG50 in 2019. After being told there was no treatment or cure, Terry brought together scientists, clinicians, families, and donors to fund and develop a gene therapy, successfully treating Michael within three years.

Terry went on to establish Elpida Therapeutics, a nonprofit biotechnology organization dedicated to developing therapies for ultra-rare pediatric diseases often overlooked because of their limited commercial potential. To date, Terry and the teams he has brought together have helped treat 17 children across three rare diseases, with programs operating across seven clinical sites in five countries. With additional programs and sites coming online in 2027, Terry continues to work alongside leading scientists, clinicians, regulators, patient organizations, and biotechnology partners worldwide, driven by one mission: to build sustainable pathways for rare disease treatments and save as many children as possible.

Michelle Tarver, MD, PhD
Director, Center for Devices and Radiological Health, FDA



Dr. Michelle Tarver is a visionary public health executive, board-certified in ophthalmology with a doctorate in epidemiology, who serves as the Director of the Center for Devices and Radiological Health (CDRH). She has spent more than 15 years as a medical device regulator, driving strategic initiatives, conducting clinical research, and changing organizational culture. Dr. Tarver has held various leadership positions while at the FDA, including the Deputy Director of the Office of Strategic Partnerships and Technology Innovation, and the Program Director of Patient Science and Engagement.

Over the course of her career, she has conducted laboratory-based and epidemiological studies, clinical trials, and surveys to capture patient preferences, as well as developed registries and patient-reported outcome measures. Dr. Tarver has extensive policy experience in crafting regulations, guidances, and conducting premarket and postmarket reviews. She most recently served as the Deputy Center Director for Transformation, steering the development, implementation, and direction

of CDRH's transformative projects and strategic initiatives. Under her leadership, CDRH has launched efforts to amplify the perspectives of people living with medical conditions, foster collaboration across the health care ecosystem, and stimulate creative evidence generation pathways.

Dr. Tarver received a BS in Biochemistry from Spelman College in Atlanta and completed the MD/PhD program at the Johns Hopkins University School of Medicine and Bloomberg School of Public Health. As a dedicated clinician, she continues to care for people living with inflammatory eye conditions.

Tamar Thompson, MS

Vice President Patient Experience & Insights, Alexion, AstraZeneca Rare Disease



Tamar Thompson, with nearly three decades of experience spanning patient advocacy, public policy, commercialization, and healthcare strategy, has dedicated her career to advancing patient-centered approaches across the life sciences ecosystem. As Vice President, Patient Experience & Insights at Alexion, AstraZeneca Rare Disease, she leads efforts to integrate patient-generated data and lived experience across the development pipeline, helping ensure patient and caregiver perspectives inform decisions related to research, development, regulatory strategy, and access. Throughout her career, Tamar has worked across industry, policy, and advocacy to address barriers to care and advance healthcare solutions that better reflect the needs of patients and communities. Tamar also serves as a mentor with the Termeer Foundation and on the board of directors of the National Comprehensive Cancer Network Foundation and Biopharma Leaders of Color.

Ashley Valentine, MRes

Co-Founder & CEO, Sick Cells



Ashley Valentine is a Co-Founder and the Chief Executive Officer of Sick Cells. Her older brother and Co-Founder of Sick Cells, Marqus Valentine, had sickle cell anemia, Hgb ss. Growing up, Ashley spent much of her childhood charming hospital playroom attendants while Marqus was frequently hospitalized for pain crises, fever, and multiple complications from SCD.

While in high school, Ashley first understood the disparities for SCD when her brother developed seizures and as a result, lost his motor skills. The family's insurance would not cover Marqus' rehabilitation, because sickle cell is an unknown chronic disease. The insurance company told the Valentine family that Marqus was considered "not disabled enough"

for medical help. Ashley and the family worked together to rehab Marqus at home. They taught him how to hold a fork again and redeveloped his ability to speak, leading to a successful recovery. This event solidified the drive in Ashley to educate others about SCD and advocate on behalf of her family and the sickle cell community.

As an adult, Ashley completed her Master's in Research Methods from the University Of Aberdeen, Scotland. She focused on disparities in healthcare for people with SCD in London. After graduate school, she worked with the University of Illinois in Chicago's sickle cell program and later transitioned into policy work in Washington, DC. While working as a policy researcher, Ashley successfully wrote sickle cell disease into part of an \$8 million Centers for Medicaid and Medicare Services (CMS) funding opportunity to address disparities for adults in the emergency department.

In 2016, Ashley moved from policy research to clinical research at Children's National Medical Center where she got a deeper understanding of clinical trials and clinical development for SCD. From there, she was able to take her learnings and passion to fulfill Marqus' dream of advocating for sickle cell disease. Ashley became the President of Sick Cells in 2017. She was able to transition into a full-time role in 2018. Since then, Ashley and her brother have achieved great milestones with the SCD community including successfully leading the SCD community in its first-ever ICER review, building coalitions to advocate for federal legislation to be signed into law, testify at the FDA about the importance of drug development, and more.

Moderator

Susan C. Winckler, RPh, Esq.
CEO, Reagan-Udall Foundation for the FDA



Susan C. Winckler, RPh, Esq., is CEO of the Reagan-Udall Foundation for the Food and Drug Administration. Prior to accepting the Foundation post, Ms. Winckler served as President of Leavitt Partners Solutions. As President and Chief Risk Management Officer for the Leavitt Partners family of businesses, Ms. Winckler advised corporate executives on policy and business matters. As CEO of the Food & Drug Law Institute, she provided attorneys, regulators, and other stakeholders with a neutral forum to address domestic and global issues. As Chief of Staff for the FDA (2007-2009), Winckler managed the

Commissioner's Office, served both Republican and Democratic commissioners as their senior-most staff adviser, analyzed complex policy challenges, and represented FDA with myriad government entities and external stakeholders. Her earlier career service included more than a decade at the American Pharmacists Association. Winckler earned a bachelor's degree from the University of Iowa College of Pharmacy and her law degree magna cum laude from Georgetown University Law Center. She is an APhA Fellow, served as an elected member of the United States Pharmacopeial Convention (USP) Board of Trustees (2015-2020, 2020-2025) and Chair of that Board from 2019 to mid-2025. In 2023, she received the Distinguished Alumni Award of the Food and Drug Administration Alumni Association and, separately, was awarded the Osterhaus Medal for Lifetime Achievement by the Univ. of Iowa College of Pharmacy.