

**RARE  
DISEASE  
DAY-2026  
FEBRUARY 23**

**FDA**

**U.S. FOOD & DRUG  
ADMINISTRATION**



**VIRTUAL PUBLIC MEETING**

*Moving forward. Looking ahead.*

**AN EVENT FOR PATIENTS**

# Welcome to FDA's Rare Disease Day 2026

## VIRTUAL PUBLIC MEETING

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### **Access the Webcast link:**

<https://www.fda.gov/news-events/fda-meetings-conferences-and-workshops/public-meeting-fda-rare-disease-day-2026-02232026>

# WELCOME

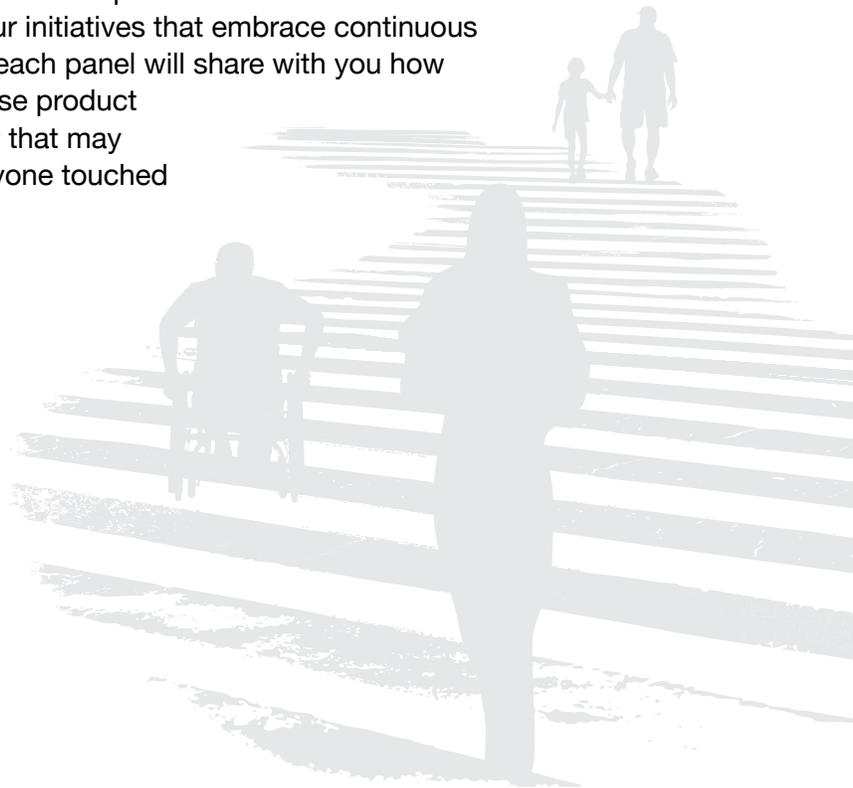
Welcome to FDA's Rare Disease Day (RDD) 2026! Our event is dedicated to patients with rare diseases, their families, care partners, and advocates.

This year, our theme is: "Moving forward. Looking ahead. An event for patients." We will share four different topics of interest to the rare disease community:

- FDA initiatives to advance medical product development for rare diseases;
- "Getting all the voices together." How the patient community can engage with FDA;
- Addressing challenges and opportunities with artificial intelligence (AI) technology; and
- Using real-world data and real-world evidence at FDA to promote product development for rare diseases.

The era of medical product development for rare diseases began with enactment of the Orphan Drug Act in 1983. It 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. Rare disease medical product development has greatly accelerated since its enactment. Core to the Orphan Drug Act is the concept of *continuous innovation*. At today's event, FDA will showcase some of our initiatives that embrace continuous innovation in the rare disease space. Today, each panel will share with you how FDA is moving forward to improve rare disease product development—and, as we look forward, how that may translate to better medical products for everyone touched by a rare disease or condition.

*Thank you for attending today's  
FDA Rare Disease Day 2026 event.  
Please take a moment to provide  
your feedback so we can continue  
to improve future events.*



# MEETING AGENDA

<p>9:00 a.m.</p>	<p><b>Welcoming Remarks</b></p> <p><b>Martin A. Makary M.D., M.P.H.</b> <i>Commissioner, U.S. Food and Drug Administration</i></p>
<p>9:10 a.m.</p>	<p><b>Overview of RDD at FDA</b></p> <p><b>Sandra Retzky, D.O., J.D., M.P.H.</b> <i>Senior Medical Advisor, RDIH</i></p> <p><b>Stacy Mathew, M.P.P.</b> <i>Health Scientist, Office of External Affairs, Office of the Commissioner</i></p>
<p>9:15 a.m.</p>	<p><b>PANEL 1: FDA initiatives to advance medical product development for rare diseases</b></p> <p><b>Session Overview</b> Experts from each FDA Center will spotlight initiatives aimed to improve product development for rare diseases.</p> <p><b>Moderator</b></p> <p><b>Amy Comstock Rick, J.D.</b> <i>Director, Strategic Coalitions for the RDIH</i></p> <p><b>Topics and Panelists:</b></p> <ul style="list-style-type: none"><li>■ <b>Center for Drug Evaluation and Research (CDER)</b> <b>Learning and Education to ADvance and Empower Rare Disease Drug Developers (LEADER 3D) Program</b></li><p><b>Andrea (Drea) Bell-Vlasov, Ph.D.</b> <i>Science Policy Analyst, CDER Rare Diseases Team</i></p><li>■ <b>Center for Biological Evaluation and Research (CBER)</b> Building on patients and caregivers input: perspectives on safety considerations and early enrollment into cell and gene therapy clinical trials for rare diseases</li><p><b>Najat Bouchkouj, M.D.</b> <i>Associate Director for Pediatrics, Office of Clinical Evaluation, CBER</i></p></ul>

# MEETING AGENDA — continued

	<ul style="list-style-type: none"><li>■ <b>Center for Devices and Radiologic Health (CDRH)</b> The role of digital health tools in accelerating and decentralizing clinical trials for devices <b>Cynthia (Cyndi) Grossman, Ph.D.</b> <i>Director, Division of Patient-Centered Development, CDRH</i></li><li>■ <b>Oncology Center of Excellence (OCE)</b> Clinical trial designs for osteosarcoma—a very rare bone cancer. Outcomes from a co-sponsored workshop between the Osteosarcoma Institute and FDA <b>Kristin Wessel, M.D.</b> <i>Medical Officer, Division of Oncology 2, Office of Oncologic Diseases, Office of New Drugs, CDER</i></li><li>■ <b>Rare Disease Innovation Hub (RDIH)</b> <b>Outcomes from the Rare Disease Innovation, Science, and Exploration (RISE) Public Workshop Series</b> <b>Amy Comstock Rick, J.D.</b> <i>Director, Strategic Coalitions for the RDIH</i></li></ul>
	<b>Q/A 15 minutes</b>
<b>10:30-10:45 a.m.</b>	<b>BREAK 1</b>
<b>10:45 a.m.</b>	<b>PANEL 2: “Getting all the voices together.” How the patient community can engage with FDA</b>  <b>Session Overview</b> 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 care partners 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.

# MEETING AGENDA

## Moderator

**Wendy Slavit, M.P.H., C.H.E.S.**

*Senior Health Scientist, Public Engagement Staff  
Office of External Affairs, Office of the Commissioner*

## Topics and Panelists

### ■ Topic 1: Thinking about product development for rare diseases with a collaborative voice

#### Panelists:

##### **ACCELERATE**

*An international multi-stakeholder organization which works at accelerating new drug development in all pediatric cancers, including osteosarcoma, with FDA and EMA as key participants along with patient advocates.*

#### Co-presenters

##### **Martha Donoghue, M.D.**

*Associate Director for Pediatric Oncology and Rare Cancers, Oncology Center of Excellence, Office of the Commissioner; and Acting Associate Director for Pediatric Oncology, Office of Oncologic Diseases, CDER*

##### **Nicole Scobie**

*Chair, ACCELERATE Board of Directors and Scientific Steering Committee member*

### ■ Topic 2: Young patient advocates for rare diseases

#### Panelists:

##### **Ann Graham**

##### **MIB (Make It Better) Agents Osteosarcoma**

*Executive Director*

##### **Maeve Smart**

##### **MIB Ambassador Agent**

*MIB NextGen team member for OsteoWarriors aged 21-26; and Medical Student, Dartmouth Geisel School of Medicine*

# MEETING AGENDA – continued

	<p><b>Liam McCarthy</b> <i>Young adult rare disease patient advocate; and Member, FDA and Clinical Trials Transformation Initiative (CTTI) Patient Engagement Collaborative</i></p> <p><b>Carter Hemion</b> <i>Young adult rare disease patient advocate; and Member, FDA and CTTI Patient Engagement Collaborative</i></p>
	<p><b>Q/A 15 minutes</b></p>
<p><b>12:00-1:00 p.m.</b></p>	<p><b>LUNCH BREAK</b></p>
<p><b>1:00 p.m.</b></p>	<p><b>PANEL 3: Addressing challenges and opportunities with artificial intelligence (AI) technology</b></p> <p><b>Session Overview</b> Over the past two years, the number of sponsor submissions with artificial intelligence and machine learning (ML) components has substantially grown. This session will provide an overview of the use of AI at FDA and how AI is being utilized in sponsor medical product submissions.</p> <p><b>Moderator</b> <b>Anindita (Annie) Saha, B.S.</b> <i>Associate Director Strategic Initiatives for the Digital Health Center of Excellence, CDRH</i></p> <p><b>Topics and Panelists</b></p> <ul style="list-style-type: none"><li>■ <b>AI tools at FDA</b> <b>Will Liu, Ph.D.</b> <i>Director of AI Governance and Implementation, CDER</i></li></ul>

# MEETING AGENDA

	<ul style="list-style-type: none"><li>■ <b>Exploring the use of artificial intelligence to support FDA regulatory review in oncology</b> <b>Gautam Mehta, M.D.</b> <i>Associate Director for Oncology Clinical Policy, OCE</i></li><li>■ <b>Use of AI in drug and biological product development</b> <b>Qi Liu, Ph.D., M.Stat.</b> <i>Associate Director for Innovation &amp; Partnership Office of Clinical Pharmacology, Office of Translational Sciences Co-chair, CDER AI Council Lead, CDER AI Review Rapid Response (R3) Team, CDER</i> <b>Johnny Lam, Ph.D.</b> <i>Associate Director for Policy Office of Therapeutic Products, CBER</i></li><li>■ <b>AI/ML-enabled medical devices: opportunities &amp; challenges in rare diseases</b> <b>Shawn Forrest, M.S.</b> <i>Digital Health Specialist, CDRH</i></li></ul>
	<b>Q/A 15 minutes</b>
<b>2:15-2:30 p.m.</b>	<b>BREAK 2</b>
<b>2:30-3:45 p.m.</b>	<b>PANEL 4: Using real-world data (RWD) and real-world evidence (RWE) at FDA to promote product development for rare diseases</b>  <b>Session Overview</b> Panelists will discuss RWD and RWE at FDA and explain how it can be leveraged to accelerate product approvals for rare diseases. Panelists will describe what is RWD and RWE, what constitutes usefulness, strengths and weaknesses, and when real-world data may generate real-world evidence. Examples will be provided during the discussion.

# MEETING AGENDA – continued

## Moderator

**Lei Xu, M.D.**

*Acting Director*

*Office of Orphan Products Development*

*Office of the Commissioner*

## Panelists:

**Marie Bradley, Ph.D., M.S.**

*Senior Advisor for Real-World Evidence*

*Office of Medical Policy, CDER*

**Pallavi Mishra-Kalyani, Ph.D.**

*Deputy Director, Division of Biometrics V*

*Office of Biostatistics, CDER*

**Rosa Sherafat-Kazemzadeh, M.D.**

*Branch Chief, General Medicine Branch 2*

*Office of Therapeutic Products, CBER*

**Charles Viviano, M.D.**

*Chief Medical Officer*

*Office of Clinical Evidence and Analysis, CDRH*

**Q/A 15 minutes**

**3:45-3:55 p.m.**

## CLOSING REMARKS

**Amy Comstock Rick, J.D.**

*Director, Strategic Coalitions for the RDIH*

# SPEAKER AND MODERATOR BIOGRAPHIES



**Andrea (Drea) Bell-Vlasov, Ph.D.**  
*Science Policy Analyst*  
*Center for Drug Evaluation and Research*  
*U.S. Food and Drug Administration*

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

Andrea spent eight years in the Center for Devices and Radiological Health where most of her work was done in the diabetes devices space; mainly for type 1 diabetes. Her time in CDRH provided significant experience in writing regulations, stakeholder outreach, clinical trial design, real-world data and evidence, pre- and post-market challenges, and much more. Her work in diabetes earned her a Samuel J. Heyman Service to America Medal in 2017. Andrea currently works in Rare Diseases policy supporting guidance development efforts and leads the **Learning and Education to ADvance and Empower Rare Disease Drug Developers** or otherwise known as the LEADER 3D initiative.

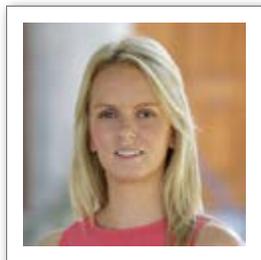
Andrea received her Ph.D. in Analytical Chemistry from the University of Michigan.



**Najat Bouchkouj, M.D.**  
*Pediatric hematologist-oncologist*  
*Associate Director for Pediatrics, Office of Clinical Evaluation,*  
*Center for Biologics Evaluation and Research*  
*U.S. Food and Drug Administration*

Najat Bouchkouj, M.D., is a pediatric hematologist-oncologist who serves as Associate Director for Pediatrics in the Office of Clinical Evaluation (OCE) at the FDA's Center for Biologics Evaluation and Research (CBER). In this role, Dr. Bouchkouj spearheads strategic regulatory, scientific, and policy initiatives that advance cellular, tissue, and gene therapy development for pediatric patients. Since joining the FDA in 2016, Dr. Bouchkouj has played a pivotal role in developing regulatory frameworks for hematologic malignancies, rare diseases, and patient engagement initiatives. Her expertise has contributed to the approval of numerous innovative cellular, tissue, and gene therapy products. She also led the development of several FDA guidance documents and serves on multiple Agency committees. Dr. Bouchkouj earned her medical degree from Damascus University and completed pediatric residency at SUNY Downstate Medical Center, followed by fellowship training in Pediatric Hematology Oncology at Children's National Medical Center. Prior to her FDA roles, Dr. Bouchkouj served as an attending physician at Boston Children's Hospital and MedStar Georgetown University Hospital. She continues to provide clinical care as a consulting oncologist at Children's National Medical Center.

## SPEAKER AND MODERATOR BIOGRAPHIES — *continued*



### **Marie Bradley, Ph.D.**

*Science Policy Analyst  
Senior Advisor for RWE, Office of Medical Policy,  
Center for Drug Evaluation and Research  
U.S. Food and Drug Administration*

Dr. Marie Bradley is a Senior Advisor for RWE in the Office of Medical Policy, Center for Drug Evaluation and Research (CDER). Her responsibilities related to RWE include serving as lead for the Advancing Real-World Evidence Program, the Real-World Evidence Subcommittee, and a portfolio of RWE demonstration projects. She also evaluates complex real-world evidence related study protocols submitted to FDA. Recently, she has been driving Agency level RWE initiatives in the Office of the Commissioner. Dr Bradley is an established advocate, speaker, and panelist across national and international platforms on RWE-related topics and participates extensively in external engagement. She is a pharmacoepidemiologist and a pharmacist with over 16 years of experience working in regulatory, government, and academic sectors in US and the UK, including 11 years at the FDA. Dr. Bradley has a Ph.D. in Pharmacoepidemiology and a Masters in Pharmacy degree from Queen's University Belfast as well as an M.Sc. in Public Health from the London School of Hygiene and Tropical Medicine.



### **Martha Donoghue, M.D.**

*Associate Director  
Pediatric Oncology and Rare Cancers, Oncology Center of Excellence,  
Office of the Commissioner  
Acting Associate Director  
Pediatric Oncology, Office of Oncologic Diseases,  
Center of Drug Evaluation and Research  
U.S. Food and Drug Administration*

Martha Donoghue, M.D. is a board-certified pediatrician and pediatric hematologist/oncologist. She is the Associate Director for Pediatric Oncology and Rare Cancers in the FDA's Oncology Center of Excellence, Office of the Commissioner and the Acting Associate Director for Pediatric Oncology in the Office of Oncologic Diseases, Center of Drug Evaluation and Research (CDER). In these roles, she oversees the implementation of pediatric regulations designed to facilitate the timely investigation of drugs and biological products for pediatric patients with cancer, supports and promotes consistency of regulatory work relating to pediatric oncology drug development across CDER and the Center for Biologics Evaluation and Research (CBER), and works with stakeholders to address challenges and foster development of drugs to treat pediatric and other rare cancers. Areas of special interest include the use of innovative clinical trial designs and use of real-world data to optimize drug development for rare cancers. Prior to joining FDA in 2009, Dr. Donoghue completed a fellowship in Pediatric Hematology and Oncology at the Children's National Medical Center

# SPEAKER AND MODERATOR BIOGRAPHIES

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.



## **Shawn Forrest, M.S.**

*Digital Health Specialist*

*Digital Health Center of Excellence (DHCoE)*

*U.S. Food and Drug Administration*

Shawn Forrest is a Digital Health Specialist in the Digital Health Center of Excellence (DHCoE) at the FDA. He served as a biomedical engineer lead reviewer and supervisor over review of diagnostic cardiovascular devices, including a variety of artificial intelligence/machine learning (AI/ML)-enabled medical devices, for the agency for 14 years. For the past five years he has helped guide the DHCoE AI/ML program developing related policy and resources for the agency. He leads the CDRH AI Standards Committee and serves as the FDA liaison to several key AI standard committees related to medical devices.



## **Ann Graham**

*Founder and Executive Director of MIB Agents Osteosarcoma Alliance*

Ms. Graham, Founder and Executive Director of MIB Agents Osteosarcoma Alliance, advocates for pediatric osteosarcoma patients. Her interest in the field stems from her own diagnosis with osteosarcoma at age 43 and subsequent treatment in a pediatric cancer center. This firsthand experience inspired her to address the critical lack of funding and awareness in pediatric cancers. In 2012, Ann founded MIB Agents to “Make It Better” for children with osteosarcoma. The organization achieves this through comprehensive patient and family programs, education for clinicians and patients, an annual conference, legislative work, and funding impactful research. Ann’s dedication is further demonstrated by her extensive involvement in numerous memberships and service roles with prominent cancer organizations, including SARC, NCCN, CAC2, and the Alliance for Childhood Cancer. She is also a recognized voice in publications and media, and a frequent speaker at significant forums like The White House (2021-2024) and industry gatherings, passionately advocating for improved outcomes for young cancer patients.

# SPEAKER AND MODERATOR BIOGRAPHIES – *continued*



## **Cynthia (Cyndi) Grossman, Ph.D.**

*Director of the Division of Patient-Centered Development,  
Center for Devices and Radiological Health  
U.S. Food and Drug Administration*

Cynthia (Cyndi) Grossman, Ph.D. is Director of the Division of Patient-Centered Development at Center for Devices and Radiological Health at the US Food and Drug Administration. She is the former Head, Real-World Research Networks, Biogen Digital Health. Prior to Biogen, Dr. Grossman was Director, Science of Patient Input at Milken Institute's FasterCures where she developed and led programs to advance patient-centered biomedical research and health care delivery. She spent a decade at the Division of AIDS Research (DAR) at the National Institute of Mental Health (NIMH), managing a grant portfolio focused on improving the lives of people living with HIV and preventing transmission.

Cyndi is an experienced leader across industry, government, non-profit and academia, and is passionate about advancing better health outcomes for all through data and bringing the lived experience of patients into decision-making. She earned her doctoral degree in clinical psychology from the University of Vermont, trained at Brown University, and has received multiple awards including Phi Beta Kappa and grants from NIH and Patient Centered Outcomes Research Institute (PCORI).



## **Carter Hemion**

Carter Hemion is a patient advocate, writer, member of the FDA & CTTI Patient Engagement Collaborative, and lifelong rare disease patient. They live with classical Ehlers-Danlos syndrome and several of its rare or rarely diagnosed co-occurring conditions. Carter is the Advocacy & Accessibility Coordinator at Stripes of Solidarity to amplify underrepresented voices in the rare disease space, equip advocates with meaningful tools, and promote equity across research, policy, and care. They also currently serve as the Community Engagement Director at Immunocompromised Association to serve immunocompromised community members by promoting education, raising awareness, and fostering community connections. Carter advocates for equitable care access and shortening the diagnostic odyssey through actions including writing, public speaking engagements, event organizing, creative expression, and meeting with policymakers.

# SPEAKER AND MODERATOR BIOGRAPHIES



## **Johnny Lam, Ph.D.**

*Associate Director for Policy  
Office of Therapeutic Products (OTP)  
Center for Biologics Evaluation and Research  
U.S. Food and Drug Administration*

Johnny Lam is an Associate Director for Policy in the Office of Therapeutic Products (OTP) in the Center for Biologics Evaluation and Research at FDA, where he conducts scientific reviews and works on policy related to jurisdiction, compliance, regenerative medicine therapies, and advanced manufacturing. He is a member of FDA's Tissue Reference Group and serves as the technical expert on the full range of compliance and policy development and implementation activities related to the regulatory review of products in OTP's purview, including human cell and tissue products, blood derivatives, devices, combination products, and cell and gene therapies. Prior to his current role, Johnny was a staff scientist and product reviewer in OTP. His regulatory portfolio included Chemistry, Manufacturing, and Controls (CMC) and product review of cellular products, tissue engineered medical products, and medical devices. He also served as the lead reviewer of numerous delivery devices for different cell and gene therapies. Johnny is a biomedical engineer by training with interests in biomaterials and developing practical microscale in vitro tools for medical and biological applications. He completed his undergraduate studies in Bioengineering at UC Berkeley and then completed graduate studies at Rice University, where he received his Ph.D. in Bioengineering. His thesis work focused on creating and evaluating injectable multi-layered hydrogel composites for cell delivery and controlled growth factor release in cartilage tissue repair.



## **Qi Liu, Ph.D., M.Stat.**

*Associate Director for Innovation & Partnership  
Office of Clinical Pharmacology (OCP), Office of Translational Sciences  
Center for Devices and Radiological Health  
U.S. Food and Drug Administration*

Qi Liu, Ph.D., M.Stat., is the Associate Director for Innovation & Partnership in the Office of Clinical Pharmacology (OCP)/ Office of Translational Sciences, CDER, FDA. She is a co-chair of CDER's AI Council and the lead of CDER AI Review Rapid Response Team. She is a member of the FDA-wide AI Policy Coordination and Planning Council. She is also on the executive board of CDER's Quantitative Medicine Center of Excellence. She helped establishing the OCP Innovative Data Analytics Program (focusing on AI, real-world data and digital health) and AI review team, and serves as the lead. She had the experience leading OCP's Physiological Based Pharmacokinetic Modeling and Simulation Oversight Board and co-leading Biologics Oversight Board. She was a co-lead initiating the Real-Time Oncology Review and Assessment Aid Pilot Programs. During her career at the FDA, Qi has participated in numerous medical product reviews to support drug development. She worked on working groups for FDA

## SPEAKER AND MODERATOR BIOGRAPHIES — *continued*

guidance documents and Manual of Policies & Procedures development. She is an Associate Editor of Clinical Translational Science and on the editorial board of five scientific journals. Before joining FDA, Dr. Liu was a senior pharmacokineticist at Merck & Co. Inc. She obtained her Ph.D. degree in Pharmaceutics and a concurrent Master's degree in Statistics from the University of Florida in 2004. In addition, she has a Master's degree in Pharmaceutics and a Bachelors' degree in Clinical Pharmacy from West China University of Medical Sciences.



### **William Liu, Ph.D.**

*Director for AI Governance and Implementation, FDA AI Internal Council  
Senior Economist, Center for Drug Evaluation and Research (CDER)  
U.S. Food and Drug Administration*

William Liu is the Director of AI Governance and Implementation at the FDA AI Internal Council and is a Senior Economist in the Center for Drug Evaluation and Research (CDER). William has led the Agency-wide effort to govern the responsible implementation and use of new enterprise AI tools and capabilities, including Elsa. He previously served as a co-chair of the CDER AI Council as well as the chair of the CDER AI Community of Practice. In his capacity as an Economist at CDER, he has advised leadership on drug and biologic pricing, pharmaceutical manufacturing, and user fee negotiations. William has a Ph.D. in Nanoscience and an M.S. in Epidemiology & Biostatistics from Northwestern University, Feinberg School of Medicine, as well as a B.S. and B.A. in Molecular Toxicology and Environmental Economics from the University of California, Berkeley.



### **Martin A. Makary M.D., M.P.H.**

*Commissioner of Food and Drugs  
U.S. Food and Drug Administration*

Dr. Marty Makary was confirmed on March 25, 2025 by a bipartisan vote of the U.S. Senate as the 27th Commissioner of Food and Drugs.

Prior to joining the FDA, Dr. Makary worked at Johns Hopkins University School of Medicine, where he was a surgical oncologist and chief of Islet Transplant Surgery. After six years on the faculty at JHU, Dr. Makary was named an endowed chair in gastrointestinal surgery, and subsequently promoted to full professor with tenure. He also served as a professor at the Johns Hopkins Carey Business School and founded the Johns Hopkins Center for Surgical Trials and Outcomes Research.

Dr. Makary is a widely published writer, having authored more than 300 peer-reviewed articles in medical journals. He is the author of three New York Times bestselling books on health care, including: "Unaccountable – What Hospitals Tell

# SPEAKER AND MODERATOR BIOGRAPHIES

You and How Transparency Can Revolutionize Health Care;” “The Price We Pay,” which examined health care costs; and which was named 2020 Business Book of the Year by the Association of Business Journalists; and “Blind Spots -- When Medicine Gets It Wrong, and What It Means for Our Health,” which presents the latest scientific research on the microbiome, food and other health topics.

Dr. Makary has led cross-disciplinary research on a range of subjects, including cancer care, obesity, frailty and psychological reserve in older patients, adverse event monitoring, the Orphan Drug Act, antimicrobial resistance, and Alzheimer’s. Of particular note, he is the co-developer of the Surgery Checklist used in many operating rooms around the world today.

Dr. Makary was the first to perform several novel surgical operations, including the first-in-the-world series of laparoscopic pancreas islet transplant operations. For his pioneering work, Dr. Makary was awarded the Nobility in Science Award from the National Pancreas Foundation. For the last 22 years, he has had an active clinical practice.

During the COVID pandemic, he and his Johns Hopkins colleagues conducted landmark antibody studies on natural immunity published in JAMA. Most recently, his research has focused on vulnerable populations in health care.

Dr. Makary has led national quality collaboratives, served on several editorial boards, and was the first editor-in-chief of MedPage Today. He has served in a leadership position at the World Health Organization Patient Safety Program, and in 2018 was elected to the National Academy of Medicine.

Dr. Makary graduated from Bucknell University and earned an M.P.H. from the Harvard T.H. Chan School of Public Health. He received his M.D. from Thomas Jefferson University and did his surgical residency at Georgetown University, completing sub-specialty surgery training at Johns Hopkins University.



## **Stacy Mathew, M.P.P.**

*Health Scientist*

*U.S. Food and Drug Administration*

Stacy Mathew is a Health Scientist at the FDA. She leads external engagement and public events for the Commissioner’s Office, the Center for Biologics Evaluation and Research (CBER), and the Center for Drug Evaluation and Research (CDER). In this role, she coordinates stakeholder meetings, webinars, and strategic communications on critical public health topics, including artificial intelligence in healthcare, vaccine confidence, chronic disease management, and medical product shortages. She also serves as the communications lead for Rare Disease Day.

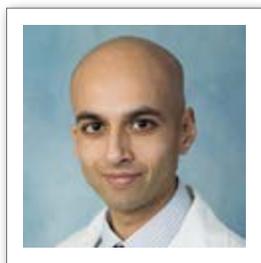
Stacy holds a Master of Public Policy degree with a specialization in Health Policy from the University of Maryland, Baltimore County.

## SPEAKER AND MODERATOR BIOGRAPHIES — *continued*



### **Liam McCarthy**

Liam McCarthy is a rare disease advocate with a personal connection to the work he does. Living with classic congenital adrenal hyperplasia (CAH), he is deeply committed to elevating patient voices and creating more inclusive, patient-centered systems. He serves as a Youth Leader for Rare Diseases International and participated in the 78th World Health Assembly to help bring attention to the experiences of young people living with rare conditions. Liam is a member of the FDA and CTTI Patient Engagement Collaborative and the NIH Patient Advisory Board, where he contributes insights to help shape research and policy through a patient lens. At Neurocrine Biosciences, he supports the CAH community by advising on outreach efforts, creating patient-focused content, and sharing his story to raise awareness and connection. He also works with the Krishnan Family Foundation as a Youth Ambassador in order to promote awareness for the rare genetic disease, Constitutional Mismatch Repair Deficiency Syndrome. Finally, he most recently got the honor of being the North American Regional Representative for the International Rare Disease Day campaign, “Raising Youth Voices.”



### **Gautam Mehta, M.D.**

*Associate Director for Oncology Clinical Policy  
Oncology Center of Excellence  
U.S. Food and Drug Administration*

Gautam is the Associate Director for Oncology Clinical Policy in the Oncology Center of Excellence at FDA, where he works to promote the consistency and efficiency of oncology regulatory review. He is the lead for Project Confirm, which provides transparency around the use of accelerated approval in oncology and co-leads the Oncology AI Program. He previously served a clinical reviewer for the nervous system, pediatrics and rare diseases team in oncology, as a team lead for the thoracic and head and neck oncology team, and as a member of CDER’s Medical Policy and Program Review Council. Prior to joining FDA, Gautam practiced as a neurosurgeon with subspecialty expertise in neurosurgical oncology and skull base tumors at the House Clinic in Los Angeles. During that period, he was chair of the Endolymphatic Sac Tumors/Audiologic Screening Guidelines Subcommittee for the Von Hippel-Lindau Alliance. He has published over 80 scientific papers and book chapters related to neuro-oncology and has served as an Associate Editor for the Journal of Neuro-Oncology and a member of the Executive Committee for the American Association of Neurological Surgeons Section on Tumors.

# SPEAKER AND MODERATOR BIOGRAPHIES



## **Pallavi Mishra-Kalyani, Ph.D.**

*Deputy Director*

*Division of Biometrics V, Office of Biostatistics,  
Office of Oncology Drugs, Center for Drug Evaluation and Research  
U.S. Food and Drug Administration*

Pallavi Mishra-Kalyani, Ph.D. is the Deputy Director of the Division of Biometrics V, Office of Biostatistics which supports Office of Oncology Drugs at the Center for Drug Evaluation and Research (CDER). Since joining the FDA in 2015, Dr. Mishra-Kalyani has contributed to the efforts to understand and address the statistical issues in oncology drug development, with a focus on novel and innovative clinical trial design and considerations for rare and pediatric tumors. In particular, she has been a key member of internal and external groups creating guidance and conducting research related to the potential use of external controls, real-world data and real-world evidence, novel endpoint development, and seamless trial designs for regulatory purposes. She has organized and participated at several statistics and oncology workshops, conferences, and working groups on these topics. Dr. Mishra-Kalyani received her doctorate in Biostatistics from Emory University, her Master's degree in Epidemiology from the T.H. Chan School of Public Health at Harvard University, and her Bachelor's degree from MIT.



## **Sandra (Sandy) Retzky, D.O., J.D., M.P.H.**

*Senior Medical Advisor*

*U.S. Food and Drug Administration*

Sandy is the Senior Medical Advisor for the Rare Disease Innovation Hub at FDA. Sandy is a pharmacist, board-certified physician, and licensed attorney.

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 as a business development executive. During part of this time, she continued to see patients on a pro bono basis at Baylor Women's Correctional Institution in Wilmington, Delaware.

In 2010, Sandy transitioned to a career in public health. To make the change, she obtained a Master of Public Health degree from Johns Hopkins Bloomberg School of Public Health in 2011 and a J.D. degree from the Delaware Law School at Widener University in 2014. Sandy joined FDA in 2016. She is a Fellow in the American College of Legal Medicine.

## SPEAKER AND MODERATOR BIOGRAPHIES — *continued*



### **Amy Comstock Rick, J.D.**

*Director of Strategic Coalitions for FDA's Rare Disease Innovation Hub  
U.S. Food and Drug Administration*

Amy Comstock Rick, J.D., is the Director of Strategic Coalitions for FDA's Rare Disease Innovation 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's goals. 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.



### **Anindita (Annie) Saha, B.Sc.**

*Associate Director for Strategic Initiatives  
Digital Health Center of Excellence,  
Center for Devices and Radiological Health  
U.S. Food and Drug Administration*

Anindita (Annie) Saha is Associate Director for Strategic Initiatives for the Digital Health Center of Excellence (DHCoE) in the Center for Devices and Radiological Health at FDA where she supports promoting innovation of digital health technologies, including those enabled by artificial intelligence. She is also serving as the acting Associate Director for Data Science and Artificial Intelligence Policy at the FDA Center for Drug Evaluation and Research to support the use of AI across the drug development lifecycle. Additionally, Annie helped incubate and continues to support to advance the science and adoption of patient input as evidence, including patient preference information (PPI), clinical outcome assessments (COAs). Previously, Annie was

# SPEAKER AND MODERATOR BIOGRAPHIES

the Director of the Partnerships team in CDRH where she oversaw a broad program portfolio, supporting several strategic partnership and regulatory science programs. Ms. Saha started as a researcher in the Office of Science and Engineering Laboratories in imaging display technologies. Ms. Saha has a Bachelor of Science in Bioengineering and Minor in History from the University of Pittsburgh.



## **Nicole Scobie**

*Chair of the ACCELERATE Board*

*Founding member and president-emeritus of Zoé4life*

Nicole Scobie, the Chair of the ACCELERATE Board and long-serving member of its Scientific Steering Committee, is a founding member and president-emeritus of Zoé4life, a Swiss non-profit supporting children with cancer, their families, and research. As a parent of a childhood cancer survivor, she advocates for better treatment access and the development of improved therapies. With extensive experience in incorporating patient perspectives into research strategies and clinical trial design, Nicole is a founding member of the ITCC and SIOPEN Advocate Committees. She serves on the board of CAC2, an international childhood cancer umbrella organization, and actively contributes to the Research & Innovation pillar of the European Branch of Childhood Cancer International. Recently she is serving in LifeArc's Strategic Advisory Board as well as the Canadian ACCESS External Advisor Board.



## **Rosa Sherafat-Kazemzadeh, M.D.**

*Pediatric Endocrinologist*

*Branch Chief for General Medicine Branch 2, Therapeutic Products (OTP),  
FDA Center for Biologics Evaluation and Research (CBER)*

*U.S. Food and Drug Administration*

Dr. Rosa Sherafat is a board-certified pediatric endocrinologist and the branch chief for General Medicine Branch 2, in the Super-Office of Therapeutic Products (OTP), in the FDA Center for Biologics Evaluation and Research (CBER). She received her medical degree from Tehran University of Medical Sciences, completed residency in pediatrics at University of Illinois Medical Center, Chicago, IL and fellowship in pediatric endocrinology in Cincinnati Children's Hospital, Cincinnati, OH. She has been an associate professor of pediatrics in the Department of Pediatrics, Medstar Georgetown University Hospital, Washington, DC (2007-2018).

Dr. Sherafat joined FDA CBER in 2018 and has served as a clinical reviewer and Team Lead in General Medicine Branch 2 for several cellular, tissue, gene therapy products and medical devices for a variety of neurology, dermatology and pulmonary indications. Dr. Sherafat served as the clinical co-chair and FDA speaker at the 70th Meeting of the Cellular, Tissue, and Gene Therapies (CTGT)

## SPEAKER AND MODERATOR BIOGRAPHIES – *continued*

Advisory Committee Meeting on Toxicity Risks of AAV Vector-Based Gene Therapy Products (September 2-3, 2021). She participated in the revision of the FDA Guidance for Industry, Long Term Follow Up after Administration of Human Gene Therapy Products (January 2020) and has represented CBER in several public meetings and workshops.



### **Wendy Slavit, M.P.H., C.H.E.S.**

*Senior Health Scientist.*

*Patient Affairs, Office of External Affairs, Office of the Commissioner  
U.S. Food and Drug Administration*

Wendy Slavit is a Senior Health Scientist in FDA's Patient Affairs, Office of Clinical Policy and Programs, Office of the Commissioner. In her role, she collaborates with patient communities, the FDA medical product Centers and other offices to incorporate patient and caregiver perspectives into FDA's work.

Ms. Slavit leads and manages FDA's **Patient Engagement Collaborative (PEC)**. The PEC is an ongoing, shared setting in which the patient community (PEC members), the FDA, and the Clinical Trials Transformation Initiative discuss many topics for improving communication, education, and patient engagement related to medical product regulation. She also focuses on health education, plain language, and health literacy through communication initiatives like the **For Patients** website and the **Patients Matter** video series.

She has almost twenty years of experience in public health, health behavior, and health education. Ms. Slavit is passionate about translating science, health research, and policy into easy to comprehend information for patients, caregivers, and the public.

She earned a Master of Public Health (MPH) from Emory University with a specialty in Behavioral Sciences and Health Education and a BA from Tufts University in Psychology. Ms. Slavit is also a Certified Health Education Specialist (C.H.E.S.).



### **Maeve Smart, B.Sc.**

*MIB Agents NextGen & Medical Student, Dartmouth College*

Maeve is a two-time osteosarcoma survivor and is eleven years No Evidence of Disease. She served as President of the MIB Agents Junior Advisory Board in 2021. She has been the Co-Lead of the OsteoWarriors Headquarters, or "HQ", at MIB Agents' annual FACTOR Conference since 2024. HQ is a camp-like experience for kids and adolescents and youth adults who have experienced osteosarcoma over two and a half days during the conference. Currently, Maeve is an MIB Agents NextGen team member for OsteoWarriors aged 21-26, who are pursuing a career in medicine or law. NextGen mentors the Junior Advisory Board and receives mentorship from medical professionals who are in the career of their choice. Maeve also serves as an MIB Ambassador

# SPEAKER AND MODERATOR BIOGRAPHIES

Agent. Ambassador Agents are trained in peer support for newly diagnosed patients with osteosarcoma. Maeve completed her undergraduate degree in Health Science at Northeastern University in 2022. She worked as a Senior Clinical Research Coordinator in the Janeway Genomics Lab at Dana-Farber Cancer Institute until 2024 before beginning medical school at Geisel School of Medicine at Dartmouth.



**Charles Viviano, M.D.**  
*Chief Medical Officer  
Office of Clinical Evidence and Analysis (OCEA),  
Center for Devices and Radiological Health  
U.S. Food and Drug Administration*

Dr. Viviano is the Chief Medical Officer in the Office of Clinical Evidence and Analysis (OCEA) in the Center for Devices and Radiological Health at FDA. In OCEA, he is primarily involved in clinical study initiatives, including utilization of real-world data across the total product lifecycle.

He previously served as the Clinical Deputy Office Director in the Office of GastroRenal, ObGyn, General Hospital, and Urology Devices (OHT3). Upon joining the FDA in 2015, he was the sole urology medical officer in CDRH, responsible for the clinical review of all urologic devices, including the first submissions for high intensity focused ultrasound devices for prostate ablation. He was also the principal investigator for an FDA-led project identifying patient preferences in prostate cancer treatment.

Prior to joining the FDA, he was an Assistant Professor in the Division of Urology at Duke University where his practice focused on general urology and men's health. Prior to Duke, he was in private practice in Connecticut. He received his medical education at the University of Connecticut and his Ph.D. in Toxicology from the University of North Carolina at Chapel Hill.



**Kristin Wessel, M.D.**  
*Pediatric Hematologist-Oncologist  
Division of Oncology 2, Office of Oncologic Diseases  
U.S. Food and Drug Administration*

Dr. Kristin Wessel is a pediatric hematologist-oncologist serving as a medical officer for the Division of Oncology 2 in the Office of Oncologic Diseases on the team dedicated to neurologic, pediatric, and rare cancers. Prior to joining the FDA, Dr. Wessel completed her pediatric residency at the University of Chicago and went on to complete her pediatric hematology-oncology training at Johns Hopkins Children's Center and the National Cancer Institute in Maryland. Dr. Wessel spent an additional year as an Advanced Clinical Fellow in the Pediatric Oncology Branch of the National Cancer Institute, where she focused on translational research in pediatric sarcomas as well as early-phase clinical trials in pediatric patients with solid tumors.

## SPEAKER AND MODERATOR BIOGRAPHIES — *continued*



### **Lei Xu, M.D., Ph.D.**

*Acting Director*

*Office of Orphan Products Development,*

*Office of Chief Medical Officer, Office of the Commissioner*

*U.S. Food and Drug Administration*

CDR Lei Xu, M.D., Ph.D., serves as the Acting Director of Office of Orphan Products Development (OOPD) in Office of Chief Medical Officer (OCMO), the Office of Commissioner, FDA. She began her FDA career in 2009 as a primary clinical reviewer in the Center for Biologics Evaluation and Research (CBER), later advancing to Branch Chief, where she oversaw approval decisions for 12 CBER products. In early 2024, she joined OOPD as a primary reviewer, where she quickly gained a strong grasp of the legal framework for orphan drugs and made meaningful contributions to OOPD's grant programs. In November 2025, she became the Acting Office Director overseeing OOPD's congressionally mandated programs and other activities that support and advance the development and evaluation of new treatments for rare diseases.

CDR Xu earned her M.D. from Central South University Xiangya School of Medicine in China and her Ph.D. in neuroscience from Yale University. She completed her neurology residency at Loyola University Chicago and is board-certified in Neurology by the American Board of Psychiatry and Neurology.

# SHINING A LIGHT ON RARE DISEASES



In observance of Rare Disease Week—February 23, 2026 to February 27, 2026—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.

*Credit: National Center for Advancing Translational Sciences*



In recognition of Rare Disease Day, CDC lit up the entrance to their Roybal Campus.

*Credit: U.S. Center for Disease Control and Prevention.*



**U.S. FOOD & DRUG  
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10903 New Hampshire Avenue, Silver Spring, MD 20993