NATIONAL ACADEMIES Sciences Engineering Medicine

Processes to Evaluate the Safety and Efficacy of Drugs for Rare Diseases or Conditions in the United States and the European Union

FDA

Jacqueline Corrigan-Curay, J.D., M.D.
Principal Deputy Center Director, Center for Drug Evaluation and Research

Jacqueline Corrigan-Curay, J.D., M.D., is the Principal Deputy Center Director in the FDA's Center for Drug Evaluation and Research (CDER) where she provides executive leadership on strategic initiatives that advance CDER's mission to deliver safe, effective and high-quality medications to the American public. Prior to taking on this role, Dr. Corrigan-Curay was the director of CDER's Office of Medical Policy leading the development, coordination, and implementation of medical policy programs and strategic initiatives, including on real-world evidence, use of technology in drug development and prescription drug promotion. Before joining FDA, she served as senior medical officer with the Immediate Office of the Director, National Heart, Lung and Blood Institute (NHLBI) at the National Institutes of Health (NIH). She also served as director of the Office of Biotechnology Activities (OBA), Office of Science Policy at NIH. Dr. Corrigan-Curay earned her law degree from Harvard Law School, her medical degree from University of Maryland School of Medicine, and a bachelor's degree in history of science from Harvard/Radcliffe College in Cambridge, MA.

Celia Witten, Ph.D., M.D.
Deputy Director, Center for Biologics Evaluation and Research

Celia M. Witten, Ph.D., M. D. is the Deputy Director of the Center for Biologics Evaluation and Research at the Food and Drug Administration (FDA/CBER). Between 2005 and 2016 she served as the Director of the Office of Cellular, Tissue and Gene Therapy at the FDA/CBER. Between 1996 and 2005 she served as Director of the Division of General, Restorative, and Neurological Devices in the Office of Device Evaluation in the Center for Devices and Radiological Health (CDRH). Previous to FDA, she worked for over 10 years as a practicing physician at the National Rehabilitation Hospital (NRH) in Washington, D.C. Her educational background includes a B.A. earned at Princeton University (Magna Cum Laude), a Ph.D. from Stanford University, and an M.D. from the University of Miami School of Medicine. In addition to her academic achievements, she is Board Certified in Physical Medicine and Rehabilitation.

Sandra Retzky, D.O., J.D., M.P.H.
Director, Office of Orphan Product Development, Office of the Commissioner

Dr. Sandra "Sandy" Retzky is the Director of the Office of Orphan Product Development (OOPD) at FDA. Sandy joined the Agency in 2016 and worked in the Center for Tobacco Products as a Medical Reviewer on applications for marketing authority of tobacco products. In 2019, Sandy became a CBER Medical Reviewer and spent several years managing many gene and cell therapy files to treat rare diseases. Sandy initially trained as a pharmacist, receiving her degree from the University of Illinois College of Pharmacy. She graduated from Midwestern University, an osteopathic medical school in Chicago. Sandy's medical credentials include board certification in Obstetrics and Gynecology, fellowship training in Urogynecology, and licensure to practice medicine in Delaware. After practicing medicine for many years, Sandy received an MBA degree from the Wharton School at the University of Pennsylvania and worked in the pharmaceutical and biotech industries for

more than a decade evaluating the commercial and clinical potential of externally sourced new medicines and negotiating licensing rights to these assets. During part of this time, she continued to see patients on a pro bono basis at Baylor Women's Correctional Institution in Wilmington, Delaware. In 2010, Sandy transitioned to a career in public health. To make the change, she obtained a Master of Public Health degree from Johns Hopkins Bloomberg School of Public Health in 2011, where she retains a faculty position, and a J.D. degree from the Delaware Law School at Widener University in 2014. Sandy is admitted to practice law in Maryland and New Jersey.

Lewis Fermaglich, M.D., M.H.A.
Medical Officer, Office of Orphan Product Development, Office of the Commissioner

Dr. Lewis Fermaglich is a board-certified pediatrician and currently a Medical Officer in the Office of Orphan Products Development (OOPD). In OOPD, he works on orphan drug and rare pediatric disease (RPD) designations, acts as a Project Officer for several Orphan Products Clinical Trials grants, and is conducting research on trends in orphan designated diseases and drugs since the enactment of the Orphan Drug Act. He received his undergraduate degree at Wesleyan University and then an MD from the University of Kentucky College of Medicine. He completed his pediatric residency at Children's National Medical Center in Washington, DC, where he was Chief Resident. After residency, he was a practicing general pediatrician for 10 years – first as a military physician, and then in private practice in Rockville, MD. Lewis came to FDA in 2017, originally assigned to the Division of Clinical Review (DCR) in the Office of Generic Drugs (OGD) as a Medical Officer.

Kerry Jo Lee, M.D.
Associate Director for Rare Diseases, Division of Rare Diseases and Medicine Genetics, Office of Rare Diseases, Pediatrics, Urological, and Reproductive Medicines, Office of New Drugs, Center for Drug Evaluation and Research

Dr. Kerry Jo Lee is the Associate Director for Rare Diseases in the Division of Rare Diseases and Medical Genetics, Office of New Drugs (OND), Center for Drug Evaluation and Research (CDER). In this role she leads the Rare Diseases Team, a multidisciplinary rare disease programming and policy team that works to promote their mission to facilitate, support, and accelerate the development of drugs and therapeutic biologics for rare diseases and serves as the program management office for CDER's Accelerating Rare diseases Cures (ARC) Program. Dr. Lee joined the FDA as a medical officer in 2014 with the former Division of Gastroenterology and Inborn Errors Products, OND, CDER. Dr. Lee then moved to a position as a clinical advisor for the Office of New Drug Policy, CDER, where she served as a lead in the areas of benefit-risk assessment, modernization efforts (including the integrated review for marketing applications), and real- world data/evidence programming before serving in her current position. Dr. Lee is a pediatric gastroenterologist/hepatologist and a graduate of Princeton University and the New York University School of Medicine with an honors degree conferred in microbiology. She completed her residency in pediatrics at the Children's Hospital of Los Angeles followed by a post-doctoral clinical fellowship in Pediatric Gastroenterology, Hepatology, and Nutrition at Columbia University College of Physicians and Surgeons in New York. Dr. Lee maintains a steadfast interest in international policy and bioethics and worked for several years at the former National Bioethics Advisory Commission on reports advising the executive branch on ethical and policy issues in both international and domestic clinical trials.

Katherine Tyner, Ph.D.
FDA Liaison to the EMA, Office of Global Policy and Strategy, Office of the Commissioner

Dr. Katherine Tyner is the FDA liaison to the EMA. Dr. Tyner joined FDA in 2007 and has held numerous positions within CDER to advance the quality, safety, and efficacy of complex drug products. In her most recent role as the Associate Director for Science in the Office of Pharmaceutical Quality (OPQ), Dr. Tyner led the OPQ Science Staff in coordinating the intersection between science, review, and policy as well as facilitating interactions among FDA Centers and Offices. Prior to her appointment as liaison, Dr. Tyner was in the Office of Science and Technology Policy within the Executive Office of the President working on efforts to increase technology transfer across the research, development, and deployment continuum.

Steffen Thirstrup
Chief Medical Officer, European Medicines Agency

Steffen Thirstrup is a medical doctor and board-certified specialist in clinical pharmacology and therapeutics. He holds a PhD in pharmacology and has a long background in clinical internal medicine with special emphasis on adult respiratory medicine. Additionally, Dr. Thirstrup was appointed adjunct professor in pharmacotherapy at the Faculty of Health Sciences, University of Copenhagen, in 2012.From 2004-09 Steffen Thirstrup worked at Danish Medicines Agency first as the Danish member of CHMP at the European Medicines Agency (EMA) for five years including 10 months as joint CHMP- and CAT-member, followed by a short period as head of Danish Institute for Rational Pharmacotherapy dealing with HTA and best practice guidelines for primary care. In 2011 Prof. Thirstrup rejoined the licensing division at the Danish Medicines Agency acting as Head of Division for Medicines Assessment and Clinical Trials. During this period Prof Thirstrup co-chaired the European Commission's working group on market access for biosimilars medicinal products and acted as key scientific contact for the managing entity of the IMI beneficiaries for the PROTECT collaboration (Pharmacoepidemiological Research on Outcomes of Therapeutics by a European ConsorTium). In March 2013, Prof Thirstrup joined the pharmaceutical consultancy company NDA Group AB as a full-time medical advisor on NDA's regulatory advisory board. In April 2014 Prof Thirstrup was appointed as director for the Regulatory Advisory Board at NDA Regulatory Services Ltd. Since June 2022 Prof Thirstrup has been the Chief Medical Officer at the European Medicines Agency, Amsterdam, The Netherlands.

Industry Perspectives

Lucy Vereshchagina, Ph.D.
Senior Vice President of Science and Regulatory Advocacy, PhRMA

Dr. Vereshchagina is senior vice president of science and regulatory advocacy at the Pharmaceutical Research and Manufacturers of America (PhRMA). In this role, Lucy leads PhRMA's science and regulatory advocacy department supporting PhRMA's policy, advocacy, and science priorities, as well as global regulatory advocacy efforts, including the International Council for Harmonization (ICH). Dr. Vereshchagina was the lead PhRMA negotiator for both PDUFA VII and BsUFA III and testified before Congress on the agreements' reauthorization. Prior to joining PhRMA in 2012, Dr. Vereshchagina spent over 12 years with the FDA & Healthcare Practice at an international law firm and the Investigational Drug Branch at the National Cancer Institute, NIH. Lucy has Master's degrees in Biochemistry and Chemistry, earned her Ph.D. in Chemistry at the Catholic University of America, and completed her post-doctoral studies in molecular biology and immunology at the Walter Reed Army Institute of Research.

Diego Ardigò, Ph.D.
Global Rare Diseases, Research & Development Head, Chiesi Group

Diego is currently the Global Head of Research & Development in Rare Diseases at Chiesi Group. Before this role, he was leading Chiesi's development projects in rare diseases and advanced therapies and has more than 20 years of experience in medical research and more than 10 in drug development, spanning from pre-clinical to commercial phase. Diego is an MD with a specialization in Internal Medicine. He obtained a PhD at the University of Parma (Italy) in cardiovascular pathophysiology and a post-doctoral fellowship in cardiovascular genomics at Stanford University (California, US). Before joining the industry, he worked at the University of Parma (Italy) in the field of cardiovascular and metabolic genomics, and as free-lance consultant for various academic institutions. He joined Chiesi in 2010, where acted as Clinical Lead in the registration of the first stem cell therapy in EU and led the cross-company team (with uniQure BV) treating the first patient with a commercial gene therapy in EU. Diego is a member and vice-chair of the board of the European Confederation of Pharmaceutical Entrepreneurs (EUCOPE) and until February 2021 served as chairman of the Therapies Scientific Committee of IRDiRC (International Rare Diseases Research Consortium), where he led the Orphan Drug Development Guidebook initiative. He is author of 50+ indexed papers and frequent speaker at international medical and industrial congresses.

E'Lissa Flores, Ph.D.
Director of Science and Regulatory Affairs, BIO

Dr. E'Lissa Flores joined the Biotechnology Innovation Organization (BIO) in 2023 as a Director of Science & Regulatory Affairs advocating for science policy initiatives that promote biomedical research advances. She currently leads several of BIO's committees include the Rare Disease & Orphan Drug, Biosimilars, ICH M4Q, and the Manufacturing committee with its various Taskforces, as well as supports the Clinical Trial Diversity committee. Previously, Dr. Flores was a Scientific Program Manager at the Health and Environmental Science Institute overseeing multi-sector committees of Genetox, ImmunoSafety, and Cardiac Safety to strategize solutions to overcome global health issues. Prior experience includes, advising philanthropic biomedical ventures in the think-tank industry including managing a $12 million grant program; formerly a NSF Science Outreach Coordinator; as well as serving on an Executive Diversity and Inclusion Committee at a major medical center. Dr. Flores received her B.S. in Biology from Stony Brook University, post-baccalaureates in Microbiology & Immunology, as well as her doctoral degree in Translational Biomedical Science from University of Rochester School of Medicine and Dentistry with an immunotoxicology focus. E'Lissa is also a

strong advocate for women and underrepresented minorities in the STEM fields engaging in many outreach and mentor programs.

Victor Martens, M.Sc.
Government Affairs Director, EUCOPE

Victor Maertens is the Government Affairs Director for the European Confederation of Pharmaceutical Entrepreneurs (EUCOPE). Victor leads EUCOPE's policy work in the area of Orphan therapies and Cell and Gene Therapy. Prior to joining EUCOPE, Victor worked for Brussels-based consultancies, providing pharmaceutical companies, trade associations, and NGOs with advocacy, strategy, and communications advice. His experience and interests include ATMPs, antimicrobial resistance, OMP, and neglected tropical diseases.

Colin Meyer, M.D.
Biogen Inc.

Dr. Colin Meyer previously served as the Chief Innovation Officer, Chief Research and Development Officer, and Chief Medical Officer for Reata Pharmaceuticals. Dr. Meyer joined the company as the second employee in 2003. While Chief Medical Officer and Chief Research and Development Officer, he led development of Skyclarys® (omaveloxolone), the first approved treatment for Friedreich's ataxia, which is a progressive, life-shortening hereditary neurological disorder. During his tenure at Reata, Dr. Meyer led strategic, analytical, operational, and field-based teams comprising product development and strategy, clinical trial design, oversight, operations, and analysis, biostatistics, research, medicinal chemistry, and medical affairs functions. Dr. Meyer received a B.S. in chemistry with specialization in biochemistry and a B.A. in biology from the University of Virginia, an M.D. from the University of Texas Southwestern Medical School, and an M.B.A. from Southern Methodist University Cox School of Business.

Huong Huynh, Ph.D.
Director of Regulatory Science, Critical Path Institute (C-Path)

Huong Huynh currently serves as the Director of Regulatory Science at Critical Path Institute (C-Path) and Professor of Practice at The University of Arizona James E. Rogers College of Law. At C-Path, she leads the development of regulatory strategies to ensure global regulatory endorsements of novel methodologies that advance clinical trials and accelerate drug development for rare disorders, neurodegenerative diseases, and vulnerable populations. At The University of Arizona, she serves as lead instructor for the course LAW 576A: Drug Discovery, Development, and in the Market as part of the Graduate Certificate in Regulatory Science. Prior to joining C-Path, Huong served as a review pharmacologist at FDA where she contributed to the development of regulatory policies and technical assessment of regulatory submissions that resulted in the approvability of over 50 drug products. In addition, Huong currently serves on the Board of the Orange County Regulatory Affairs (OCRA) group and has previously served on the Boards and committees of the FDA Alumni Association, San Diego Regulatory Affairs Network (SDRAN), FDA Women of CDER, Association of Women in Science (AWIS) – San Diego Chapter, and the National Postdoctoral Association (NPA). Huong has also served as Session Chair at DIA Global 2023; as Session Panelist at RAPS Convergence 2022; as the Associate Members liaison to the FDA Alumni Association (FDAAA) Board of Directors; and as a remote volunteer on the San Diego Regulatory Affairs Network (SDRAN) Education Committee. Huong received her PhD in pharmacology and completed an NIH-funded postdoctoral training in signal transduction.

Sabrina Paganoni, M.D., Ph.D.
Co-Director, Massachusetts General Hospital Neurological Clinical Research Institute (NCRI)

Sabrina Paganoni, MD, PhD, is an Associate Professor of PM&R at Harvard Medical School / Spaulding Rehabilitation Hospital. She is also the Co-Director of the Neurological Research Institute at the Massachusetts General Hospital and physician scientist at the Healey & AMG Center for ALS. Her research focuses on clinical trials and therapy development for ALS. She has served as PI of several ALS clinical trials and has been using novel trial designs, novel endpoints, and digital technology tools to innovate the way investigational products are tested in ALS. She is the co-PI of the HEALEY ALS Platform Trial, the first platform trial for ALS in the world. She recently reported the positive results of the CENTAUR trial and is the co-Chair of the global PHOENIX trial. Her research has been funded by the NIH, non-profits, and industry; she published more than 100 peer-reviewed manuscripts and received several awards for her work including the 2021 Top 10 Clinical Research Achievement Award.

Lakiea Bailey, Ph.D.
Executive Director, Sickle Cell Community Consortium

Dr. Lakiea Bailey is a sickle cell disease advocate, educator and research scientist. Diagnosed with sickle cell disease at age three, she has become a passionate advocate for those living with rare diseases and is committed to serving as a voice of encouragement and empowerment within the sickle cell community. Despite the devastating symptoms of sickle cell, Dr. Bailey was determined to complete her educational goals, earning a Bachelor degree in Biochemistry and Molecular Biology in 2001 and a Doctorate degree in Molecular Hematology and Regenerative Medicine in 2012. During the course of her education, Dr. Bailey was named a Southern Regional Education Board (SREB) Doctoral Scholar, was the recipient of multiple honors and awards, including the Fisher Scientific Award for Overall Excellence in Biomedical Research, the Medical College of Georgia Alumni Association Award, the Georgia Reagents University Leadership Award and was inducted in the Alpha Upsilon Phi honor society. She believes that through hard work, diligence, patience and faith, even the seemingly most impossible obstacles can be overcome.