REAGAN-UDALL FOUNDATION FOR THE FDA

Scientific Advancements in Gene Therapies: Opportunities for Global Regulatory Convergence

Hybrid Public Meeting
September 4, 2024; 10AM - 4PM (eastern time)

Presenters & Panelists

Hildegard Büning, PhD
Hannover Medical School

Dr. Hildegard Büning is Professor of Infection Biology of the Gene Transfer and Deputy Director of the Institute of Experimental Hematology at Hannover Medical School (Germany). She served as President of the European Society of Gene and Cell Therapy (www.ESGCT.eu, 2018-2022), as Member of the Board of Directors of the American Society of Gene & Cell Therapy (https://asgct.org/, 2021-2024) and is the Scientific Secretary of the German Society for Gene Therapy (www.dg-gt.de/, since 2014). She studied Biology and obtained her PhD (Dr. rer. nat.) at the Ludwig-Maximilians-University Munich (Germany) in 1997. Since then, she is active in the field of adeno-associated virus (AAV) vectors with a particular focus on AAV-host interactions and vector development. Aiming to improve efficacy and safety of AAV vectors for gene therapy, she is tailoring vector tropism and immunogenicity using both rational design and library-based approaches. Photo Copyright Information: © MHH, Stefan Zorn

Cecelia Calhoun, MD, MPHS, MBA
Yale University School of Medicine

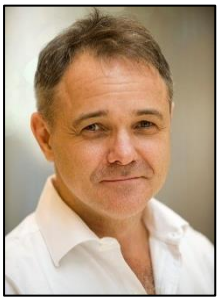
Dr. Cecelia Calhoun is an Assistant Professor of Medicine and Pediatrics at Yale University School of Medicine where her clinical and research expertise center on the care of persons with Sickle Cell Disease (SCD) She also serves as the Medical Director of the Adult Sickle Cell Program at Smilow Cancer Hospital.

Dr. Calhoun uses mixed methods to find solutions to the educational and healthcare obstacles critical to the longevity of adolescents with sickle cell disease. She has dedicated her career to the design and implementation of evidence-based interventions that promote successful transition from youth to adult care for the sickle cell population. As an NIH funded investigator, she collaborates with her hematology colleagues across the nation to use Implementation Science methods to improve outcomes for patients with sickle cell disease throughout their lifespans.

Dr. Calhoun graduated from the University of Michigan with a Bachelor of Arts degree in Afro-American Studies and Medical Doctorate from Wayne State University. She continued her training at Michigan State University as a pediatric resident, then fellowship at Washington University School of Medicine where she also

completed a Master of Population Health Sciences. She received her MBA as one of three, inaugural Pozen-Commonwealth Fund Fellows in Minority Health Leadership at Yale University School of Management.

Jeremy Farrar, MD, PhD
World Health Organization



Dr. Jeremy Farrar has been the Chief Scientist at the WHO since May 2023. In that role he helps ensure WHO is committed to science and evidence and that innovation and high-quality health products, policies, and services are available equitably to everyone, everywhere.

Jeremy is a clinician scientist who before joining the WHO was between 2013-2023 Director of the Wellcome Trust. Between 1996-2013 he was Director of the Clinical Research Unit Hospital for Tropical Diseases in Ho Chi Minh City Viet Nam. Dr Farrar trained in neurology and infectious diseases in London, Edinburgh, Oxford and in Melbourne. He has a PhD in Immunology from the University of Oxford in partnership with the University of California in San Francisco and has over 600 publications. He is a Fellow of the Academy of Medical Sciences UK, the National Academies USA, the European Molecular Biology Organization and a Fellow of The Royal Society.

Tony Ho, MD
Pivotal Life Sciences

Dr. Tony Ho is a Senior Science Partner at Pivotal Bioventure. Previously, he served as Executive Vice President and Head of R&D at CRISPR Therapeutics from 2017 to 2021, where he was responsible for global research and development across all therapeutic areas. During his tenure, Dr. Ho built a world-leading R&D platform, leading to the development of over ten product pipelines and the world's first approved CRISPR product, Casgevy. Additionally, he and his team advanced three allogeneic CAR-T therapies (CTX110 for CD19, CTX120 for BCMA, and CTX130 for CD70) and the first CRISPR-edited pancreatic islet progenitor cells (VCTX210 and VCTX211) from research to clinical trials.

Before joining CRISPR Therapeutics, Dr. Ho was the Senior Vice President and Head of Oncology Integration and Innovation at AstraZeneca, where he oversaw the development and commercialization of Lynparza® (olaparib) Imjudo and Imfinzi® (durvalumab). Prior to AstraZeneca, he led the Neurology and Ophthalmology divisions at Merck and was the Co-Founder and Chief Scientific Officer of Neuronyx.

Dr. Ho earned his M.D. from Johns Hopkins University and his B.S. in Electrical Engineering from UCLA. He has published over 80 papers across various fields and currently serves as an Adjunct Associate Professor of Neurology at the University of Pennsylvania and an Assistant Professor of Neurology at Johns Hopkins University.

Maneesha Inamdar, PhD
Institute for Stem Call Science and Regenerative Medicine

Professor Maneesha Inamdar is a stem cell and developmental biologist conducting research at Bangalore, India. Her group uses human stem cells, gene editing and animal models to study cardiovascular and blood development, to understand congenital defects and devise regenerative strategies.

Dr. Inamdar pioneered human embryonic stem cell derivation and use in India, providing stem cells that represent the Indian genetic diversity, are eligible for use globally and have been distributed worldwide. Through her association with various international stem cell organizations and with the WHO Expert Committee, Prof. Inamdar has contributed to several guidance and policy documents on stem cells and gene editing. She heads or serves on several scientific review, funding and ethics committees. While furthering discovery and training, she is deeply involved in science outreach, education and public engagement. Her recent article in the journal *Nature* was highlighted for its call to bring equity and accessibility through global research standards.

Prof. Inamdar is the Director of inStem (Institute for Stem Cell Science and Regenerative Medicine) and Senior Professor at JNCASR (Jawaharlal Nehru Centre for Advanced Scientific Research), Bangalore. She is a fellow of the Indian Academy of Sciences, the Indian National Science Academy and a J C Bose National Fellow, with several awards and honors.

Eric Karikari-Boateng, MS
Food and Drugs Authority (Ghana)

Eric Karikari-Boateng is currently the Director of the Centre for Laboratory Services and Research at the Food and Drugs Authority (FDA) Ghana.

He is a pharmacist by training and holds a master's degree in Pharmacy (Pharmacology and Toxicology) with over 24 years' experience in Quality Control and Quality Assurance of pharmaceuticals and Biopharmaceutical products.

He is a Secondary Reviewer of CMC dossiers covering molecules 3 – Drug Substance and Drug Product (small molecules and biologics). Eric is a member of the Technical Coordinating Committee (TCC) of the African Vaccines Regulatory Forum (AVAREF).

David Liu, PhD
Broad Institute of MIT and Harvard

David R. Liu is the Richard Merkin Professor, director of the Merkin Institute of Transformative Technologies in Healthcare, and vice-chair of the faculty at the Broad Institute of MIT and Harvard. He is also the Thomas Dudley Cabot Professor of the Natural Sciences at Harvard University and an HHMI investigator. Liu's research integrates chemistry and evolution to develop new therapeutics, including genome editing technologies such as base and prime editing, as well as other engineered proteins and *in vivo* delivery vehicles. His innovations have been pivotal in developing treatments for genetic diseases and are used globally in at least 10 clinical trials. Liu, who graduated first in his class from Harvard University and earned his PhD from UC Berkeley, has published over 260 papers and holds more than 100 patents. He has received numerous awards, including the 2022 King Faisal Prize in Medicine and the 2024 Jacob and Louise Gabbay Award, and has been recognized as a leading global thinker and influential figure in the life sciences. Liu is also a founder of several biotechnology companies.

Julie Makani, MD, PhD, FRCP, FTAAS
Muhimbili University of Health and Allied Sciences
Tanzania High Commission to the UK

Professor Julie Makani is a physician scientist in Tanzania, Principal Investigator in the Sickle Cell Programme at Muhimbili University www.muhas.ac.tz and Sickle Pan African Research Consortium (SPARCO) Clinical Coordinating Centre (CCC), SickleInAfrica http://www.sickleinafrica.orghttp://www.sickleinafrica.org/. She is Provost Visiting Professor at Imperial College London, UK. She is science advisor at the Tanzania High Commission in the UK.

She received the 2011 Royal Society Africa Award on genomic research for sickle cell disease http://www.youtube.com/watch?v=sd17odE1YLs. She is Fellow of the Royal College of Physicians, UK, Tutu Leadership www.alinstitute.org, and the Tanzania Academy of Sciences.

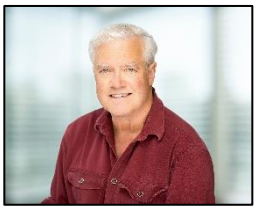
Peter Marks, MD, PhD
Center for Biologics Evaluation and Research, FDA

Dr. Marks serves as the Director of the Center for Biologics Evaluation and Research (CBER) at the Food and Drug Administration. The center is responsible for assuring the safety and effectiveness of biological products, including vaccines, allergenic products, blood, and blood products, and cellular, tissue, and gene therapies. Marks and center staff have committed themselves to facilitating the development of biological products and providing oversight throughout the product life cycle.

Dr. Marks received his graduate degree in cell and molecular biology and his medical degree at New York University. Following this, he completed an Internal Medicine residency and Hematology/Medical Oncology fellowship at Brigham and Women's Hospital in Boston, where he subsequently joined the attending staff as a clinician-scientist and eventually served as Clinical Director of Hematology.

He then moved on to work for several years in the pharmaceutical industry on the clinical development of hematology and oncology products prior to returning to academic medicine at Yale University where he led the Adult Leukemia Service and served as Chief Clinical Officer of Smilow Cancer Hospital. He joined the FDA in 2012 as Deputy Center Director for CBER and became Center Director in 2016. Dr. Marks is board certified in internal medicine, hematology, and medical oncology, and is a Fellow of the American College of Physicians. In 2022, he became a Member of the National Academy of Medicine, one of the highest honors in the fields of health, science, and medicine.

Mike McCune, MD, PhD
Bill & Melinda Gates Foundation



Dr. Mike McCune is Head of the HIV Frontiers Program at the Bill & Melinda Gates Foundation and a Professor Emeritus of Medicine at the University of California, San Francisco. After studies at Harvard College (AB), Cornell University Medical College (MD), and the Rockefeller University (PhD), he started to treat patients with HIV disease as a resident in internal medicine at UCSF from 1982-1984 and has been involved in the HIV/AIDS research field ever since, working as a postdoctoral fellow at Stanford, as Scientific Director of two biotechnology companies that he co-foundexd (SyStemix in 1988 and Progenesys in 1991), as the Chief of the Division of Experimental Medicine (which he founded) at UCSF, and as founding PI and Senior Associate Dean of the Clinical and Translational Sciences Institute at UCSF. In recent years, he has been focused on the discovery and development of safe, effective, accessible, affordable, and acceptable in vivo curative interventions for HIV and sickle cell disease.

Kwasi Nyarko, PhD
WHO Regional Office for Africa (WHO-AFRO)

Dr. Kwasi A. Nyarko, is the Coordinator, African Vaccine Regulatory Forum (AVAREF) Secretariat, a regulatory and ethics capacity building platform of the World Health Organization (WHO), African Regional Office (AFRO). He has over 23 years' experience as a regulator of health products, including vaccines, and chemicals from the Government of Canada. He has experience in all aspects of the product innovation life cycle ranging from preclinical to clinical trials, pre-market and post-market assessment of the safety, quality and efficacy, development and implementation of regulatory policy and best practices supporting authorization. Kwasi is the focal person for Vaccine Research and Development, Regulation and Safety within the Vaccine Preventable Diseases (VPD) program. Dr. Kwasi leads the AVAREF Secretariat in supporting the National Regulatory Authorities (NRAs) and National Ethics Committees (NECs), including capacity building. Kwasi has led multiple teams responsible for assessing risks posed by chemical substances to human health and the environment, development of regulatory science policy frameworks, worked on establishing trusted environments for innovation and development of a wide range of regulatory frameworks for products such as vaccines, biosimilars and biotherapeutics. Kwasi was Health Canada's representative to the WHO-AVAREF from 2008 to 2016/17. Dr. Nyarko and the team at AVAREF are working towards the operationalization of the regulatory oversight for clinical trials in the African Medicines Agency (AMA).

Jimi Olaghere
Gene Therapy Recipient

Jimi Olaghere is a CasGevy recipient who has developed a keen interest in the advancements of Cell and Gene therapies, since his transformative participation in the groundbreaking clinical trial.

He is passionate about the positive impact these therapies can have on patients in need and has become a staunch advocate for increased accessibility of gene therapies for SCD patients worldwide.

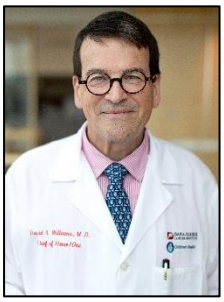
Sol Ruiz, PhD
Spanish Medicines Agency

Dr. Sol Ruiz is Head of Biologics, Biotechnology and Advanced Therapies at the Spanish Medicines Agency. She has a PhD in Biology (Immunology). She is a member of the CAT (Committee for Advanced Therapies) and a co-opted member of the CHMP (Committee for Human Medicinal Products) at the EMA (European Medicines Agency) since 2007. She has been the chair of the BWP (Biologics Working Party) at the EMA from 2014 until February 2023.

John Tisdale, MD
National Heart, Lung, and Blood Institute, NIH

Dr. John Tisdale is a senior investigator and chief of the Cellular and Molecular Therapeutics Branch of the National Heart, Lung, and Blood Institute (NHLBI) at NIH. Dr. Tisdale's research focuses on developing curative strategies for sickle cell disease through transplantation of allogeneic or genetically modified autologous hematopoietic stem cells. An elected member to the American Society for Clinical Investigation, Dr. Tisdale is the recipient of the Ernest Beutler Lecture and Prize of the American Society of Hematology. His awards include the American Society of Gene and Cell Therapy's Jerry Mendell Award for Translational Science, the George C. Marshall Innovation Leadership Award, and the Public Health Service Outstanding Service Medal. Dr. Tisdale has over 250 publications. He was the Principal Investigator on the gene therapy trial of lovo-cel for SCD that was FDA approved in 2023.

David Williams, MD
Harvard Medical School



Dr. David Williams is the Chief of the Division of Hematology/Oncology, and the Leland Fikes Professor of Pediatrics at Harvard Medical School. His laboratory has been continuously funded by the NIH since 1986. He has published over 400 peer-reviewed manuscripts and textbook chapters. He formerly served on the NIH RAC and GTSAB. His basic research has focused on hematopoiesis and his laboratory has identified the molecular basis for three rare human diseases due to mutations of GTPases RAC2, RHOH and most recently SEPTIN6. His patents led to FDA-approved drugs (Neumega® and Retronectin®). He served as the coordinating investigator for the pivotal trial for eli-cel®, approved by the FDA in 2023. Dr. Williams is a past President of the International Society of Experimental and the American Society of Hematology. He has received numerous is an elected member of the National Academy of Medicine of the National Academy of Sciences, Medicine and Engineering.

Moderator

Susan C. Winckler, RPh, Esq.
Chief Executive Officer, Reagan-Udall Foundation for the Food and Drug Administration

Susan C. Winckler is CEO of the Reagan-Udall Foundation for the Food and Drug Administration. The Foundation is the non-profit organization created by Congress to advance the mission of the FDA. Prior to accepting the Foundation post in May of 2020, 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, industry leaders, and consumers with a neutral forum to address domestic and global issues. As FDA Chief of Staff from 2007-2009, Ms. Winckler managed the Commissioner's office; served as his/her senior staff adviser; analyzed policies; and represented FDA before myriad government and external stakeholders. She simultaneously led FDA's Offices of Legislation, External Relations, Public Affairs, and Executive Secretariat. As APhA Vice President Policy/Communications and Staff Counsel, she served as the association's lead spokesperson and senior liaison to Congress, the executive branch, state associations, and allied groups. Ms. Winckler earned a bachelor's degree from the University of Iowa College of Pharmacy and her juris doctorate *magna cum laude* from Georgetown University Law Center. She is an APhA Fellow, an elected member and Chair of the United States Pharmacopeial Convention (USP) Board of Trustees (2015-2020, 2020-2025), a member of the Purgo Scientific, LLC board, and a member of the Virginia Commonwealth University School of Pharmacy National Advisory Council.