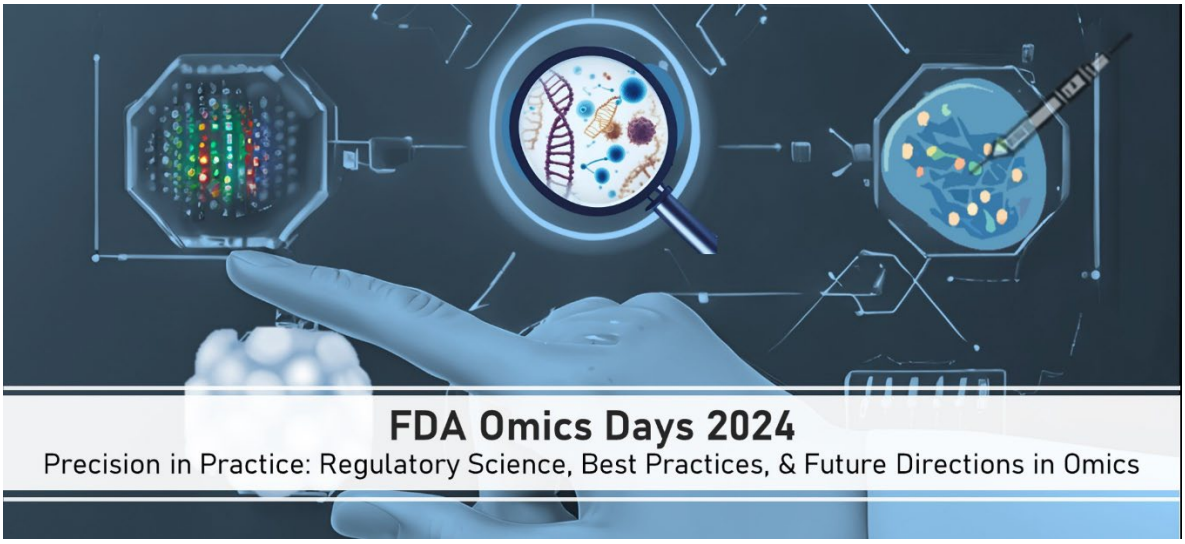

September 12, 2024

WELCOME

9:00AM – 9:05AM ET

Lerna Uzasci, PhD
Co-chair, FDA Omics Days 2024
Biologist/Lead Reviewer, Division of Microbiology Devices, Office of In Vitro Diagnostics, Office of Product Evaluation and Quality, Center for Devices and Radiological Health (CDRH), US Food and Drug Administration

Dr. Uzasci received her Ph.D. in Chemical Biology from Johns Hopkins University in 2013, followed by post-doctoral training at the National Institutes of Health. In 2014, she began her career at the U.S. Pharmacopeia, where over the years, she took on multiple positions that focused on the development and evaluation of reference standards. In 2022, Dr. Uzasci transitioned to the FDA, where she now serves as a Lead Reviewer for microbiology devices within the Office of In Vitro Diagnostics at the Center for Devices and Radiological Health.

OPENING REMARKS

9:05AM – 9:15AM ET

Robert M. Califf, MD
Commissioner, US Food and Drug Administration

Dr. Robert M. Califf was confirmed as the 25th Commissioner of Food and Drugs. As Commissioner, Dr. Califf oversees the full breadth of the FDA portfolio and execution of the Federal Food, Drug, and Cosmetic Act and other applicable laws. This includes assuring the safety, effectiveness, and security of human and veterinary drugs, vaccines and other biological products for human use, and medical devices; the safety and security of our nation's food supply, cosmetics, dietary supplements, products that give off electronic radiation; and the regulation of tobacco products. Dr. Califf has had a long and distinguished career as a physician, researcher, and leader in the fields of science and medicine. He is a nationally recognized expert in cardiovascular medicine, health outcomes research, health care quality, and clinical research, and a leader in the growing field of translational research, which is key to ensuring that advances in science translate into medical care. This is Dr. Califf's second stint as Commissioner. He also served in 2016 as the 22nd Commissioner. Dr. Califf is a graduate of Duke University School of Medicine. He completed a residency in internal medicine at the University of California, San Francisco and a fellowship in cardiology at Duke.

KEYNOTE 1

9:15AM – 10:00AM ET

Restoring trust: The need for precision medicine in infectious diseases, public health, and vaccines
Archana Chatterjee, MD, PhD

Dean, Chicago Medical School; Senior Vice President for Medical Affairs, Rosalind Franklin University of Medicine and Science

Dr. Archana Chatterjee joined Rosalind Franklin University in April 2020 and is Dean of the Chicago Medical School and Senior Vice President for Medical Affairs (the first woman and person of color to serve in these roles). For the previous 7 years, she served as Professor and Chair of the Department of Pediatrics (the first woman and person of color to serve as such) and Senior Associate Dean for Faculty Development at the University of South Dakota Sanford School of Medicine (USD SSOM). Prior to that, she was at Creighton University School of Medicine (CUSOM) where she advanced through the academic ranks to tenured Professor, served as the Division Chief for 6 years, and the Hospital Epidemiologist at the Children's Hospital and Medical Center in Omaha, NE for 13 years. Dr. Chatterjee has been elected/selected to serve on several national Advisory Boards and Committees including the US Food and Drug Administration's Vaccines and Related Biological Products Advisory Committee, the Association of American Medical Colleges' Group on Women in Medicine and Science, the Pediatric Infectious Diseases Society Board of Directors, and the American Board of Pediatrics Subboard of Pediatric Infectious Diseases. Dr. Chatterjee has focused some of her scholarly effort on collaborative projects related to faculty and leadership development as well as DEI initiatives, participating in presentations at national conferences and publishing her work in high-impact, peer-reviewed journals. Trained as a pediatric infectious disease specialist, Dr. Chatterjee has practiced in her field for over 20 years, conducted over 120 clinical trials, published over 100 peer-reviewed articles, 26 invited review articles, 31 book chapters and one book.

BREAKOUT SESSION 1: Changing landscape of precision medicine and pharmacogenomics

10:15AM – 11:45PM ET

Moderator

Paula Hyland, DPhil, MPH

Co-chair, FDA Omics Days 2024

Senior Staff Fellow and Genomics Lead, Division of Applied Regulatory Science, Office of Clinical Pharmacology, Center for Drug Evaluation and Research (CDER), US Food and Drug Administration

Paula Hyland, D.Phil., M.P.H. was trained in molecular biology and molecular epidemiology in the UK. She is currently a Senior Staff Fellow and Genomics Lead in the Division of Applied Regulatory Science, Center for Drug Evaluation and Research (CDER), FDA. Her job functions include genomics support for reviews in rare diseases and research focusing on the identification of biomarkers of drug response and toxicity using different omic technologies. Before joining the FDA in September 2017, Paula worked at the National Cancer Institute, NIH, in the Division of Cancer Prevention from 2009 to 2011, and in the Genetic Epidemiology, and the Integrative Tumor Epidemiology Branches in the Division of Cancer Epidemiology and Genetics from 2012 to 2017. Her work experience extends from in vivo and in vitro molecular biology studies evaluating the efficacy and adverse effects of drugs to omic-based discovery research in cancer susceptibility and etiology. Paula is a center representative for CDER for the FDA Omics Working Group and is co-chair of the organizing committee for 2024 FDA Omics Days.

Identification of modifiers and predictors of disease severity in X-linked adrenoleukodystrophy

Stephan Kemp, PhD

Full professor of Inherited Neurometabolic Diseases and Newborn Screening at the Amsterdam University Medical Center – University of Amsterdam

Dr. Kemp is a full professor at Amsterdam UMC, University of Amsterdam, specializing in X-linked adrenoleukodystrophy (ALD) and inherited neurometabolic diseases. With more than 25 years of experience, he has contributed extensively to ALD research (>120 publications & book chapters, >12,500 citations). As project leader, Dr. Kemp led the development of a boys-only screening algorithm for ALD as part of the Dutch newborn screening program, leading to nationwide screening from October 1, 2023. His research group collaborates with ALD physicians and researchers worldwide, emphasizing open communication and intensive collaboration. Dr. Kemp founded and am the editor of the open access worldwide ABCD1 variant registry and disease information platform www.adrenoleukodystrophy.info, a comprehensive resource in five languages with over 200,000 visitors per year for physicians and patients. He is actively involved in patient organizations, serving on medical and scientific boards in Europe and the USA, and as a board member of ALD

Connect (USA). Dr. Kemp's research team excels in translational research, bridging basic science and clinical investigation, resulting in transformative publications that shape the field of ALD.

The molecular twin artificial intelligence (AI) platform in precision oncology

Dan Theodorescu, MD, PhD

Director, Samuel Oschin Comprehensive Cancer Institute, Cedars-Sinai Medical Center

Theodorescu has emerged as an international leader in translational cancer research. His pioneering application of computational biology led to the discovery of genes that drive bladder and other cancers, while providing novel biomarkers and therapeutic targets and the foundations for personalized/precision therapeutic approaches such as the Molecular Twin Precision Oncology Platform (MT-POP) and the COXEN principle. He has led the discovery and development of a "first in class" RalGTPase inhibitor as a new therapeutic in several human cancer types. His lab was the first to demonstrate that loss of the Y chromosome in cancer makes tumors more aggressive by allowing them to evade the immune system and that this advantage becomes a liability by increasing their sensitivity to immune checkpoint inhibitors which has broad implications in cancer medicine since many tumors lose the Y chromosome. has been elected to societies including the American Society for Clinical Investigation (ASCI), Association of American Physicians (AAP), the American Surgical Association (ASA) and the National Academy of Medicine (NAM). He is an Honorary Fellow of the American Association for the Advancement of Science (AAAS). Dr. Theodorescu's presentation will discuss the advantages of multi-omic profiling of tumor and host in biomarker development; the use and utility of AI in biomarker development; and how the development of parsimonious biomarker panels can democratize precision oncology globally.

Building an atlas of variant effects to advance precision genetic medicine

Douglas M. Fowler, PhD

Professor, Department of Genome Sciences, University of Washington

Dr. Douglas M. Fowler holds three academic positions at the University of Washington (UW): Professor of Genome Sciences, Adjunct Professor of Bioengineering, and Member of the Brotman Baty Institute for Precision Medicine. Since 2019, Dr. Fowler has served as the director of the Center for the Multiplex Assessment of Phenotype, a National Human Genome Research Institute Center of Excellence in Genome Sciences. He co-directs the Center for Actionable Variant Analysis and is a founder and current executive committee member of the Atlas of Variant Effects Alliance. The Alliance consists of a diverse group of ~500 experimentalists, computational biologists, clinicians and others with the shared vision of determining the effect of every possible variant in every gene in the human genome. Dr. Fowler's work has focused on developing and implementing new technologies to address difficult problems in genomics. He is a global leader in high-throughput, sequencing-based assays; his lab has deep expertise in large-scale experimental approaches and computational analyses. He has led the use of large-scale variant effect data to interpret the clinical consequences of human genetic variation. Dr. Fowler holds a bachelor's degree in chemistry cum laude, with departmental honors, from Northwestern University and a Ph.D. in chemistry from the Scripps Research Institute.

POSTER SESSION 1

11:45AM – 1:15PM ET

Presentations in-person; selected pre-recorded poster talks for virtual attendees.

BREAKOUT SESSION 2: Omics applications on safety and efficacy for product development

1:15PM – 2:45PM ET

Moderator

Jessica Hastie, PhD

Research Reviewer, Division of Bacterial, Parasitic, and Allergenic Products, Office of Vaccines Research and Review, Center for Biologics Evaluation and Research (CBER), U.S. Food and Drug Administration

Dr. Jessica Hastie obtained her Ph.D. in Microbiology from the University of Iowa. During her doctoral studies, she studied how bacteria sense their environment. After earning her Ph.D. in 2015, she joined Dr. Paul Carlson's lab at the FDA as an Oak Ridge Institute for Science and Education Postdoctoral Fellow (ORISE) to study *Clostridioides difficile* pathogenesis. In 2019, she transitioned to an FDA staff fellow position and started performing regulatory review on a variety of products related to the microbiome in addition to research. She continues to perform this role as a staff scientist.

A multi-omics systems vaccinology resource to develop and test computational models of immunity

Bjoern Peters, PhD
Professor, La Jolla Institute for Immunology

Starting as a PhD student in 2000, Dr. Peters has worked on the development and validation of tools to analyze and predict which parts of a pathogen, allergen, or cancer cell are targeted by immune responses. Identifying these specific molecular targets of immune responses, called epitopes, recognized by diseased individuals opens a path toward developing diagnostics, vaccines, and therapeutics. The tools the Peters lab develops aim to reduce the experimental effort required to identify these targets. The second research area of the lab is the identification of differences between immune cells in individuals with divergent disease outcomes. The Peters lab uses single cell multi-omics to characterize how immune cells from diseased individuals differ from healthy individuals. These cells are isolated using disease-specific epitopes (or reagents based on them), so epitope-identifying algorithms developed in the lab directly aid in the disease-focused work. This research helps understand how the disease develops and identifies potential targets for interventions to treat or prevent the disease. Finally, the Peters lab is deeply involved in developing community standards for knowledge representation to promote interoperability and re-use of data. Dr. Peters will present the community resource his group developed for comparing Bordetella pertussis booster responses and to host annual contests for predicting patients' vaccination outcomes. He will report on their experiences with the first two rounds of the prediction contest, the biological insights gained on pertussis vaccinations, and the challenges encountered and addressed when running prediction contests.

Screening DNA adducts of harmful chemicals: The known and the unknown

Robert J. Turesky, PhD
Masonic Cancer Center and Department of Medicinal Chemistry, University of Minnesota, Minneapolis

Dr. Rob Turesky is a Professor in the Department of Medicinal Chemistry at the University of Minnesota. He received his Ph.D. in nutrition and food science from M.I.T. Before his current position, Dr. Turesky served as Group Leader of the Biomarkers Unit, Nestlé Research Center, Switzerland (1986 – 2000); Division Director of Chemistry, National Center for Toxicological Research, United States Food and Drug Administration, AR (2000 – 2004); and Principal Investigator, Wadsworth Center, New York State Department of Health (2004 – 2013). His research investigates the biochemical toxicology mechanisms of potential cancer-causing agents in the environment, tobacco, foods, cosmetic dyes, and traditional herbal medicines. Dr. Turesky uses mass spectrometry (MS) approaches to identify and measure these chemicals, their metabolites, protein- and DNA-adduction products in experimental laboratory animals, cell culture, and human biospecimens. His laboratory has developed novel technologies and MS-based approaches to detect DNA adducts in formalin-fixed paraffin-embedded tissues and exfoliated urinary cells, which are underutilized in cancer biomarker research. The lab is developing DNA adductomics, new MS technologies to simultaneously screen for DNA adducts from a wide array of genotoxicants, providing comprehensive data on genome-damaging chemicals contributing to cancer. Dr. Turesky will present his development of a novel untargeted screening method using wide-selected ion monitoring tandem mass spectrometry coupled to nanoflow liquid chromatography and an Orbitrap mass spectrometer to identify previously unreported DNA adducts in laboratory animals and humans.

Sensitivity of endogenous virus detection using high-throughput genome sequencing

Sandra Fuentes, PhD
Laboratory of Retroviruses, Division of Viral Products, Office of Vaccines Research and Review (OVRR), Center for Biologics Evaluation and Review (CBER), U.S. Food and Drug Administration

Dr. Sandra Fuentes obtained her Ph.D. in Pathobiology from Pennsylvania State University in 2010. For her dissertation she investigated the functions of the respiratory syncytial virus (RSV) phosphoprotein and small hydrophobic protein. In 2016 she completed her postdoctoral fellowship at FDA where she contributed to the development of a luciferase reporter gene based microneutralization assay for RSV and investigated the antibody repertoires of RSV infected and vaccinated individuals to identify protective epitopes. She is currently a Microbiologist at the FDA where she contributes to the development of reference materials for the evaluation of HTS platforms aimed at the detection of adventitious viruses in vaccines. Dr. Fuentes will present her work evaluating HTS potential for endogenous virus detection on multiple Illumina sequencing platforms using a model of simian foamy retrovirus (SFV) integration in a human cell line.

Xenotransplant multiomics

Jef D. Boeke, PhD

Sol and Judith Bergstein Director, Institute of System Genetics, and Professor, Department of Biochemistry and Molecular Pharmacology, NYU Langone Health

Dr. Boeke is the founding director of The Institute for Systems Genetics at NYU Langone Health and known for foundational work on mechanistic and genomic aspects of retrotransposition. He is a pioneer of synthetic genome construction, as he synthesized the first artificial yeast chromosomes de novo and leads an international consortium that built the highly engineered genome of the first synthetic eukaryote, Yeast 2.0, which synthesized 17 synthetic chromosomes to a common design, and invented genome scrambling. He has also constructed a number of synthetic metabolic pathways and introduced them into foreign genetic backgrounds, like fungus to yeast, bacterial to yeast, human to yeast, and microbial to mammalian. Using Big DNA technology to build mammalian gene loci in yeast and then delivering those loci and their variants to stem cells, Dr. Boeke and his team are also working to understand the "instruction manuals" that specify how human genes are expressed in the context of the Dark Matter Project. Boeke has founded or cofounded several biotechnology companies, and his lab developed a highly automated RT-PCR workflow and software infrastructure that was central to a COVID testing pipeline deployed by a company he helped found. His academic training was at Bowdoin College (A.B. in Biochemistry) and Rockefeller University (Ph.D. in Molecular Biology). He did postdoctoral studies with Gerald Fink at the Whitehead Institute. Dr. Boeke will present his work on very deep omic profiling of samples from decedents who have undergone xenotransplantation of kidneys and hearts, as well as developing clinical grade PCR and sequencing tests for detection of porcine exogenous as well as endogenous viruses. His findings suggest potential value in developing diagnostic tests both for early detection of rejection signals as well as ongoing monitoring of porcine and human pathogens to be deployed in large scale deployment of xeno-organs for clinical use.

BREAKOUT SESSION 3: Leveraging omics for surveillance, emerging threats, and One Health

3:00PM – 4:30PM ET

Moderator

Carlo Mercado, PhD

Co-lead, FDA Omics Working Group's Omics One Health Subgroup
Biologist Reviewer, Division of Animal Bioengineering and Cellular Therapies, Office of New Animal Drug Evaluation, Center for Veterinary Medicine (CVM), US Food and Drug Administration

Dr. Carlo Mercado is the current Acting Branch Chief for the Veterinary Research Branch in the Division of Applied Veterinary Research, Office of Applied Science, Center for Veterinary Medicine (CVM). He regularly serves as a Biologist Reviewer in CVM's Division of Animal Bioengineering and Cellular Therapies, and also co-leads the FDA Omics One Health working group. As a reviewer, Dr. Mercado evaluates submissions that support the development of intentional genomic alterations in animals, which are used in a variety of different applications. After receiving a Bachelor of Science in Forensic Chemistry-DNA Analysis from Towson University, Dr. Mercado completed his Ph.D. in Human Genetics and Genomic Medicine from the University of Maryland School of Medicine.

Standards for NGS-based biosurveillance

Scott A. Jackson, PhD

Group Leader, Complex Microbial Systems, National Institute of Standards and Technology (NIST)

Scott Jackson joined NIST in May of 2014 after 11 years as a principal investigator at FDA-CFSAN. At CFSAN, his research focused on characterizing the global genomic diversity of enteric pathogens, with applications for food safety, bioforensics and public health. At NIST, Scott is the founder and leader of the Complex Microbial Systems Group. In this current role, Scott is leading efforts to improve NGS-based measurements of complex microbial communities (a.k.a. microbiomes) with applications in biosurveillance and infectious disease diagnostics. Scott performed his graduate studies in the biochemistry and biophysics departments at The University of Maryland and Johns Hopkins University, respectfully, where he focused on the evolution of mobile genetic elements. Scott performed his undergraduate studies in Chemistry and Geology at the University of South Carolina. Dr. Jackson will present on the NIST partnership with stakeholders from industry, academia, and other government agencies (including FDA, CDC, DHS, EPA, and DOD) to develop measurement assurance tools (standards) for pathogen detection as it applies to infectious disease diagnostics, environmental biosurveillance, biothreat surveillance, and biomanufacturing quality control.

Beyond the COVID-19 pandemic: Opportunities and next steps for wastewater based epidemiology

Helena M. Solo-Gabriele, PhD

Professor, University of Miami

Helena Solo-Gabriele is a Professor of Environmental Engineering at the University of Miami where she teaches courses in water quality, environmental analysis, environmental engineering microbiology, and water and wastewater treatment. Her area of research expertise is in problems that relate human health to the environment. Through this line of research, she evaluates the transport of microbes in the environment and assesses infectious disease risk. Most recently her team utilized wastewater-based measurements of the virus that causes COVID-19 to track the transmission of disease within the University of Miami, within Miami Dade County Public Schools, within the University Hospitals and within Miami Dade County. Most recently her team has expanded workflows to evaluate the transmission of additional illnesses through measurements at regional wastewater treatment plants. Dr. Solo-Gabriele will present the applications of wastewater for tracking COVID-19 and its use in evaluating other non-respiratory illnesses such as Mpox and Candida auris. Her presentation will include workflows for expanding wastewater based epidemiology beyond SARS-CoV-2.

Advancing public health surveillance through wastewater-based genomics

Daniel Cornforth, MS, PhD

Data Scientist, Centers for Disease Control and Prevention

Dan Cornforth has a background in public health, mathematical modeling, and microbial genomic analysis and currently serves as the lead for the Data Analytics and Visualization unit in CDC's National Wastewater Surveillance System (NWSS). Dan holds a BSc in Mathematics from the University of Texas at Austin, as well as an MSc from the University of Oxford and PhD from the University of Edinburgh, both in biology, where he focused on the mathematical modeling of the transmission and evolution of bacterial pathogens. He then completed a postdoctoral fellowship in microbiology at the University of Texas at Austin before becoming a research scientist at Georgia Tech, where his research centered on bacterial gene expression in human infections. Dan joined CDC as an Epidemic Intelligence Service officer, supporting the National Syndromic Surveillance Program, and later transitioned to his current role in wastewater analysis at NWSS. At this conference, Dr. Cornforth will explore how wastewater genomics can enhance public health surveillance, offering early warnings for emerging variants of concern. The CDC's NWSS collaborates with over 30 laboratories nationwide to sequence SARS-CoV-2 in wastewater samples, providing insights into circulating variants.

An overview of Cyclosporiasis surveillance and genotyping in the United States

Lauren Ahart, MPH

Epidemiologist, Centers for Disease Control and Prevention

Lauren Ahart is a surveillance epidemiologist in Parasitic Diseases Branch (PDB) at the Centers for Disease Control and Prevention (CDC). Lauren began her CDC career in 2017 as an ORISE Fellow on CDC's Division of Foodborne, Waterborne, Environmental Diseases (DFWED), where her projects and work were focused on antimicrobial resistance in foodborne pathogens. In 2020, she joined PDB to work on cyclosporiasis surveillance, outbreak investigations, and response activities for PDB. In addition to her work on cyclosporiasis, she also assists with surveillance and technical support for epidemiologic investigations of trichinellosis. She will present the epidemiology and surveillance of cyclosporiasis in the U.S. and describe the current genotyping tool. This presentation will highlight recent outbreak investigations and demonstrate how genotyping data have been used to inform epidemiologic cluster and FDA traceback investigations.

CLOSING REMARKS

4:30PM ET

Alexis Norris, PhD

Co-chair, FDA Omics Working Group

Bioinformatics Reviewer, Division of Animal Bioengineering and Cellular Therapies, Office of New Animal Drug Evaluation, Center for Veterinary Medicine (CVM), US Food and Drug Administration

Dr. Norris joined the FDA as a bioinformatics reviewer in 2018. Prior to that, she completed her PhD and postdoctoral training at Johns Hopkins School of Medicine where she used genomics, transcriptomics,

proteomics, and metabolomics to study human cancer and psychiatric disorders. At CVM, she evaluates data, including omics data, submitted to support new animal drug applications for intentional genomic alterations (IGAs) in animals.