Jessica Chery

CMC Reviewer
Gene Therapy Branch, FDA

Dr. Chery received her PhD in Molecular Biology, Cell Biology, and Biochemistry from Brown University in 2014. Her PhD thesis characterized a previously unstudied zinc finger protein identified by the lab, elucidating its function in DNA packaging and regulation of acetylation marks implicated in cancers such as breast cancer and leukemia.

Dr. Chery did post-doctoral research at Harvard Medical School (HMS)/Massachusetts General Hospital where she identified novel small molecules with therapeutic potential for Ebola virus. While there, she collaborated on projects developing small molecules such as antisense oligonucleotides (ASOs) and RNAi as therapeutic modalities for metabolic diseases. Continuing with her interest in translational virology/immunology, she became a research fellow at Dana Farber Cancer Institute (DFCI). At HMS/DFCI, she developed a gene therapy approach for Very Early Onset Inflammatory Bowel Disease (VEO-IBD) using CRISPR and viral vector technologies.

After her postdoc fellowship, Dr. Chery became a full-time CMC reviewer at the FDA in 2018.

Anna Kwilas

CMC Team Lead
Gene Therapy Branch, FDA

Dr. Kwilas received her Ph.D. in Biomedical Science from The Ohio State University in 2010 with an emphasis in Molecular Virology & Gene Therapy and Translational Science. She performed her graduate research at The Research Institute at Nationwide Children's Hospital examining the potential application of respiratory syncytial virus as a gene therapy vector for the treatment of cystic fibrosis.

Dr. Kwilas performed her post-doctoral research at the National Cancer Institute in the Laboratory of Tumor Immunology and Biology investigating the efficacy of modified vaccinia virus Ankara and adenovirus-based cancer vaccines alone and in combination with other approved and investigational cancer therapeutics.

In 2015, Dr. Kwilas received the Interagency Oncology Task Force Fellowship. She began conducting research at FDA involving the generation of safer vector producing cells with the use of CRISPR/Cas9 genome editing technology and participating in gene therapy product CMC review. Since May 2016, Dr. Kwilas has been a full-time gene therapy CMC reviewer at the FDA. In 2019, Dr. Kwilas became a Team Lead in the Gene Therapy Branch focusing on the regulation of gene therapy products incorporating genome editing.

Peter Marks

Director

Center for Biologics Evaluation and Research, FDA

Peter Marks received his graduate degree in cell and molecular biology and his medical degree at New York University and completed Internal Medicine residency and Hematology/Medical Oncology training at Brigham and Women's Hospital in Boston. He has worked in academic settings teaching and caring for patients and in industry on drug development. He joined the FDA in 2012 as Deputy Center Director for CBER and became Center Director in 2016.

Matthew Porteus

Matthew Porteus MD, PhD is the Sutardja Chuk Professor of Definitive and Curative Medicine and a Professor in the Department of Pediatrics, Institute of Stem Cell Biology and Regenerative Medicine and Maternal-Child Health Research Institute at Stanford. His primary research focus is on developing genome editing as an approach to cure disease, particularly those of the blood (such as sickle cell disease) but also of other organ systems as well. He received his undergraduate degree at Harvard in History and Science where his honors thesis studied the recombinant DNA controversy of the 1970s. He then completed his MD and PhD training at Stanford, clinical training in Pediatric Hematology/Oncology at Boston Children's Hospital, and post-doctoral research training with Noble Laureate David Baltimore at CalTech. He works as an attending physician on the Pediatric Hematopoietic Stem Cell Transplant service at Lucile Packard Children's Hospital where he cares for children under going bone marrow transplantation for both malignant and non-malignant diseases. His goal is to combine his research and clinical interests to develop innovative curative therapies. He served on the 2017 National Academy Study Committee of Human Genome Editing and currently serves on the Scientific Advisory Board for WADA on Cell and Gene Doping and the NIH NexTRAC advisory committee evaluating the emergence of new technologies. He has been a scientific founder of CRISPR Tx and an academic founder of Graphite Bio and serves on several SAB's.

David Scott

David Scott is a co-founder, leader, and technologist passionate about building future-looking technologies to expand the reach of genomic medicines. He enjoys building and working with interdisciplinary teams at the intersection of large-scale computing, biotechnology, and therapeutic development to accelerate the path of breakthrough technology from bench to bedside.

David co-founded Arbor Biotechnologies to harness the bacterial and viral protein diversity for next-generation genome editing technologies to enable best- or first-in-class genomic medicines. As Head of Research through May 2021, David led Arbor's scientific team in the development of the company's high-throughput discovery engine and the discovery of an industry-leading portfolio of next-generation CRISPR gene editing technologies. Subsequently, as Head of Innovation, David sourced technologies and capabilities to expedite the company's preclinical and clinical development of genetic medicines. Prior to Arbor, David completed his PhD at MIT in the lab of Feng Zhang at the Broad Institute, where he explored the early applications of CRISPR systems for genome editing.

Shengdar Tsai

Dr. Tsai is an Assistant Member in the Department of Hematology at St. Jude Children's Research Hospital. His lab's research focuses on developing genome editing technologies for therapeutics, with a special interest in editing human HSCs for treatment of hemoglobinopathies such as sickle cell disease and T-cells for cancer immunotherapy. In 2020, he was chosen as one of the American Society for Gene and Cell Therapy Outstanding New Investigators.

His group has recently developed CHANGE-seq, a state-of-the-art, sensitive, unbiased, high-throughput method for defining the genome-wide activity of genome editors (Lazzarotto et al. Nature Biotechnology 2020). Previously, he has led the development of methods for high-throughput genome editing with TALENs (Reyon and Tsai et al. Nature Biotechnology 2012), CRISPR-Cas genome editors with improved specificity by dimerization (Tsai et al. Nature Biotechnology 2014), widely adopted methods to define the genome-wide specificity of CRISPR-Cas nucleases such as GUIDE-seq (Tsai and Zheng et al. Nature Biotechnology 2015) and CIRCLE-seq (Tsai et al. Nature Methods 2017 and Lazzarotto et al. Nature Protocols 2018).

Dr. Tsai completed a postdoctoral fellowship at Massachusetts General Hospital & Harvard Medical School, Ph.D. in Functional Genomics and M.S. in Bioinformatics from North Carolina State University, and B.S. from the University of Michigan.

Gilean McVean

Gil McVean is a Founder of and Chief Scientific Officer at Genomics plc. He has a background in statistical and population genetics and played leading roles in the International HapMap and 1000 Genomes Projects, as well as advisory roles in projects including the UK Biobank and Genomics England. He was the founding director of Oxford University's Big Data Institute and is a Fellow of both the Royal Society and the Academy of Medical Sciences.

Gang Bao

Dr. Gang Bao is the Foyt Family Professor and Chair of the Department of Bioengineering, Rice University. He is also a CPRIT Senior Scholar in Cancer Research and the Director of Nanomedicine Center for Nucleoprotein Machines at Rice. Dr. Bao received his undergraduate and Master's degrees from Shandong University in China, and his PhD degree from Lehigh University in the US. Dr. Bao is a Fellow of the American Association of Advancement in Science (AAAS), American Physical Society (APS), American Society of Mechanical Engineers (ASME), American Institute for Medical and Biological Engineering (AIMBE), and Biomedical Engineering Society (BMES).

Dr. Bao's current research is focused on the development of genome editing and nanomedicine tools and approaches for biological and disease studies, including the design, validation and optimization of engineered nucleases such as CRISPR/Cas9 for genome editing, and multifunctional magnetic iron oxide nanoparticles for *in vivo* imaging and drug/gene delivery. These approaches have been applied to the diagnosis and treatment of chronic diseases such as cancer, and the development of genome editing approaches for treating single-gene disorders including sickle cell disease and cystic fibrosis.

Luca Pinello

Luca Pinello is a computational biologist and leader in developing computational methods for functional genomics, genome editing and single cell technologies. He holds a Ph.D. in Mathematics and Computer Science from University of Palermo, Italy. He is currently an Associate Pathologist at Massachusetts General Hospital (MGH) and an Associate Professor of Pathology at Harvard Medical School. He is also part of the MGH Center for Cancer Research and an Associate Member of the BROAD Institute of MIT and Harvard.

He has developed several foundational computational tools in the field of genome editing for the design (CRISPRme, CRISPRitz, PrimeDesign), quantification (CRISPResso 1 and 2), and analyses of coding and non-coding tiling screens (CRISPRO, CRISPR-SURF). He was awarded one of the first NIH R35 Genomic Innovator Awards, a prestigious grant supporting highly innovative researchers working on important problems in genomics.

Laura Sepp-Lorenzino

Laura Sepp-Lorenzino, Ph.D. joined Intellia Therapeutics in 2019 as Chief Scientific Officer and is responsible for Research and Early Development. Intellia is harnessing CRISPR-based technologies to revolutionize the future of medicine. Laura previously held leadership positions at Vertex, Alnylam and Merck. She serves in the Board of Directors of Taysha Gene Therapies, the Alliance for Regenerative Medicine and the Oligonucleotide Therapeutics Society, and on the Scientific Advisory Boards for Thermo Fisher Scientific, the U.K. Nucleic Acid Therapies and Arsenal Capital Partners. She received her professional degree in Biochemistry from the University of Buenos Aires, Argentina, and both her M.S. and Ph.D. in Biochemistry from New York University.

Kelly Frazer

Dr. Frazer is a Professor in the Department of Pediatrics at UCSD School of Medicine, Chief of the Division of Genome Information Sciences, and Director of the UCSD Institute for Genomic Medicine. The goal of her research is to understand genetic predisposition to complex diseases starting in childhood but spanning the whole age spectrum. Over the past eight years, the Frazer lab has systematically derived and characterized a unique collection of iPSC lines from 222 individuals – referred to as iPSCORE (iPSC Collection for Omic Research). iPSCORE is currently being used to conduct genotype – molecular phenotype correlations. In this workshop Dr. Frazer will discuss the impact of both inherited genetic variants and somatic mutations on iPSC molecular phenotypes.