

Speaker Biographies



Graça Almeida-Porada, MD, PhD

Professor, Regenerative Medicine; Director, Fetal Research and Therapy Center
Wake Forest Institute for Regenerative Medicine

Dr. Graça Almeida-Porada is a Professor of Regenerative Medicine and Director of the Fetal Research and Therapy Program at Wake Forest Institute for Regenerative Medicine. She holds appointments in the Departments of Internal Medicine and Biomedical Engineering at the Wake Forest School of Medicine. Dr. Almeida-Porada holds an MD and a PhD in Pathology from ICBAS, University of Porto, and trained in Hematology/Transfusion Medicine at Centro Hospitalar do Porto. Her research focuses on the development of cellular and gene delivery platforms to treat genetic and immune-mediated diseases. She is particularly interested in improving the outcome of stem cell and gene delivery in fetal and neonatal patients with genetic diseases, and developing immunomodulatory therapies for patients with immune-mediated disorders. She is the Co-founder of the International Fetal Transplantation and Immunology Society (IFeTIS). Dr. Almeida-Porada holds several patents and has authored more than 200 scientific works including papers, abstracts, and book chapters.

Disclosures: None



Philip John (P.J.) Brooks

Deputy Director, Office of Rare Diseases Research
NIH National Center for Advancing Translational Sciences (NCATS)

Dr. Philip John (P.J.) Brooks is the Deputy Director in the NIH National Center for Advancing Translational Sciences (NCATS) Office of Rare Diseases Research (ORDR). Dr. Brooks received his Ph.D. in neurobiology from the University of North Carolina at Chapel Hill. Dr. Brooks is interested in accelerating clinical trials in rare diseases by moving beyond “one disease at a time” approaches, including gene editing and oligonucleotide therapies. He is also the coordinator of the NIH Common Fund’s Somatic Cell Genome Editing (SCGE) program (<https://commonfund.nih.gov/editing>) and the Interdisciplinary Scientific Committee Chair for the International Rare Diseases Research Consortium <https://irdirc.org/>.

Disclosures: None



R. Alta Charo, JD
Professor Emerita of Law & Bioethics
University of Wisconsin – Madison

R. Alta Charo is professor emerita of law and bioethics at the University of Wisconsin, where she taught reproductive rights law, biotechnology policy and public health law for over 30 years. She has also worked in government at the congressional Office of Technology Assessment and the FDA, and served on President Clinton's National Bioethics Advisory Commission. Charo has been elected to the NAM, the AAAS and the Amer. Acad. of Arts & Sciences, and co-chaired reports on stem cell research and on genome editing for the National Academies. At present, she consults on science policy and ethics for several companies and co-chairs the safety, security and social responsibility unit of BioMADE, a consortium of industrial biotechnology companies and research institutes.

Disclosures: Consultant, Johnson & Johnson Consultant, BioMADE Consultant, Colossal, Inc. Consultant, Vertex, Inc.



Agnieszka Czechowicz, MD, PhD
Assistant Professor of Pediatrics
Stanford University

Agnieszka Czechowicz spent a decade on the Farm as a Stanford undergraduate, medical student and graduate student and completed her PhD work with Prof. Irv Weissman one of the great leaders in stem cell biology. As a physician-scientist, subsequently did clinical training in Boston, completing her residency in Pediatrics at the prestigious Boston Children's Hospital and pursued subspecialty training in Pediatric Hematology/Oncology at the Dana Farber Cancer Institute while simultaneously conducting postdoctoral research with Prof. Derrick Rossi and Prof. David Scadden. Her primary clinical interest is in bone marrow failure and aplastic anemia, and in other diseases commonly necessitating stem cell transplantation. She has done pioneering work showing that hematopoietic stem cell depletion is a critical component to donor hematopoietic stem cell engraftment, and multiple pre-clinical and clinical therapies are in development based upon her studies. Dr. Czechowicz is a strong physician-scientist and advocate of translational research, she is passionate about mentoring and training future generations of physicians and scientists, and is very supportive of helping diverse trainees on various traditional and non-traditional career paths.

Disclosures: None



Anna David, PhD, FRCOG

Professor and Consultant in Obstetrics and Maternal Fetal Medicine
Institute for Women's Health, University College London

Dr. Anna David is a Professor and Honorary Consultant in Obstetrics and Maternal Fetal Medicine at UCL Hospital in London. She was appointed as Director of the UCL Elizabeth Garrett Anderson Institute for Women's Health in 2018. The Institute takes a life course, holistic approach to women's health, providing a co-ordinated strategy to clinical care, research and education.

Clinically Anna specializes in fetal medicine, congenital disease, fetal growth restriction and prevention of preterm birth. Her research is developing prenatal therapies for obstetric conditions and congenital disease in the fetus, using genetic and regenerative medicine. In 2019 she co-led the implementation of fetal surgery for spina bifida in the UK. The surgery is now specialist commissioned by NHS England.

Anna is part of the BOOSTB4 consortium performing the first clinical trial of in utero stem cell transplantation for osteogenesis imperfecta. She led development of the first standardized Maternal and Fetal Adverse Event Terminology: MFAET version 1.0 for use in clinical trials of pregnancy interventions. This terminology aims to transform the conduct of trials to test new maternal and fetal therapies, making them much safer for pregnant women and their babies.

Disclosures: I do consulting work for Esperare Foundation, Geneva, Switzerland, a private not-for-profit developing a prenatal therapy for congenital X-Linked Hypohidrotic Ectodermal Dysplasia (XLHED).



Nalin Gupta, MD, PhD

Professor, Neurological Surgery & Pediatrics
University of California San Francisco

Dr. Nalin Gupta has been a pediatric neurosurgeon at the University of California, San Francisco since 2000. His laboratory research focuses on relationship of tumor microenvironment in the central nervous system, with a focus on mouse models. Dr. Gupta has clinical experience with cell-based and gene delivery to the CNS for genetic defects in children, and he is the primary neurosurgeon at UCSF for MOMS clinical trial evaluating fetal surgical treatment for myelomeningocele.

Disclosures: Consulting agreements with the following companies: Axovia Therapeutics, BridgeBio Therapeutics, Encoded Therapeutics, Sana Therapeutics



Ying Huang, PhD

Pharmacologist, Office of Tissues and Advanced Therapies/CBER
U.S. Food and Drug Administration

Dr. Ying Huang joined FDA/CBER/OTAT/DCEPT in 2004 and is a Pharm/Tox master reviewer responsible for the review of regulatory submissions for cell and gene therapies, including genome editing and edited products on oncology and non-oncology diseases. She is the FDA Topic Leader in the ICH S12 Expert Working Group on Non-clinical Biodistribution Studies for Gene Therapy Products. Prior to the FDA, Dr. Huang received her PhD degree in Pharmacology and Toxicology at the University of Toronto followed by an NIH IRTA fellowship, before she became a senior scientist at former Genetic Therapy Inc., a Novartis Company.

Disclosures: None



Barbara Koenig, PhD

Professor Emeritus of Bioethics and Medical Anthropology
University of California San Francisco

Barbara A. Koenig, Ph.D., is Professor Emeritus of Bioethics and Medical Anthropology at the University of California, San Francisco. She served until 2021 as Director of the UCSF Program in Bioethics. Previously, Prof. Koenig was the founding executive director of the Center for Biomedical Ethics at Stanford University; she created and led the Bioethics Research Program at the Mayo Clinic in Rochester, Minn. She received her Ph.D. from the University of California, Berkeley and San Francisco joint program in Medical Anthropology.

Disclosures: None



Billie Lianoglou, MS
Genetic Counselor
University of California San Francisco

Billie Lianoglou is a genetic counselor who specializes in prenatal diagnosis and counseling. She works closely with perinatologists to educate families on potential genetic risks for fetal abnormalities or complications. Ms. Lianoglou supports the clinical trials of the UCSF Center for Maternal Fetal Precision Medicine. Working with genetic specialists Dr. Mary Norton and Dr. Teresa Sparks she has been counseling families regarding exome sequencing studies exploring the value of prenatal exome sequencing for indications including non-immune hydrops fetalis and structural differences. Ms. Lianoglou also works together with fetal surgeon Dr. Tippi MacKenzie facilitating patient recruitment and education on both the in utero stem cell transplantation study for alpha thalassemia major as well as the in utero enzyme replacement for in born errors of metabolism. As a part of these project, Ms. Lianoglou manages the international registries of patients found to have a prenatal diagnosis with these conditions. Ms. Lianoglou earned a master's degree in genetic counseling from Icahn School of Medicine at Mount Sinai.

Disclosures: None



Tippi MacKenzie, MD
Professor of Surgery
University of California San Francisco

Dr. Tippi MacKenzie is a Professor of Surgery at the University of California, San Francisco and the Director of the Eli and Edythe Broad Institute for Regeneration Medicine. She is a pediatric and fetal surgeon who is focused on developing better ways to diagnose and treat genetic diseases before birth. She runs a translational research lab examining fetal immunology and maternal-fetal tolerance, with the ultimate goal of inventing new fetal therapies for patients with genetic diseases or pregnancy complications. She recently co-founded the Center for Maternal-Fetal Precision Medicine, with the aim of accelerating the processes that link basic research to clinical trials to improve maternal, fetal, and neonatal health. Tippi has moved two fetal molecular therapies from the lab to the clinic as phase 1 clinical trials after obtaining FDA approval: in utero hematopoietic stem cell transplantation to treat fetuses with alpha thalassemia and in utero enzyme replacement therapy in fetuses with lysosomal storage disorders. Her research has been supported by the National Institutes of Health, the March of Dimes, the California Institute for Regeneration Medicine, and the Burroughs-Wellcome Fund. Tippi has been awarded the Jacobson Award by the American College of Surgeons for her innovative work and is a member of the American Society for Clinical Investigation.

Disclosures: Tippi MacKenzie is on the SAB of Acrigen and has received research funding from Ultragenyx, Novartis, and BioMarin.



William Peranteau, MD
Associate Professor of Surgery
The Children's Hospital of Philadelphia

Dr. Peranteau is an Associate Professor of Surgery at the University of Pennsylvania and the inaugural holder of the Adzick-McCausland Distinguished Chair in Fetal and Pediatric Surgery at The Children's Hospital of Philadelphia (CHOP). In addition to a clinical pediatric and fetal surgeon, Dr. Peranteau is the co-director of the Center for Fetal Research at CHOP where he runs a translational research lab focusing on prenatal cell and gene therapies. He has led teams on some of the initial studies demonstrating the feasibility of in utero CRISPR-mediated gene editing in relevant animal models and, together with Dr. Alan Flake, he has led preclinical animal studies of in utero hematopoietic stem cell transplantation which are foundational for ongoing clinical trials. His research is/has been supported by the National Institutes of Health, including the NIH Director's New Innovator Award, as well as the American College of Surgeons, the Gerber Foundation, and the Institute for Translational Medicine and Therapeutics and the Orphan Disease Center at the University of Pennsylvania.

Disclosures: None



Christopher Porada, PhD
Professor
Wake Forest Institute for Regenerative Medicine

Dr. Christopher Porada received a bachelor's degree from Colgate University in molecular biology and a PhD from the University of Nevada, Reno in Molecular and Cellular Pharmacology and Physiology, performing thesis work on in utero gene therapy (IUGT) under the tutelage of Dr. Esmail Zanjani, a pioneer and world leader in the field of fetal therapies. He is currently a Professor at the Wake Forest Institute for Regenerative Medicine. Dr. Porada's research focus over the last 25 years has been to develop safer and more efficient means of accomplishing gene transfer into clinically relevant cell types in vivo and achieving immunological tolerance to the therapeutic transgene. The ultimate goal is to use this knowledge to develop safe, effective treatments for monogenic diseases like hemophilia, which could be administered shortly after, or prior to, birth. Dr. Porada performed some of the first studies assessing and quantifying the risk of modification of the fetal germline as a result of IUGT. In addition to studies on direct in vivo gene delivery, Dr. Porada has spent over 10 years studying stem cell-based gene therapy, employing HSC and MSC as delivery vehicles for a variety of marker and therapeutic transgene cassettes in small and large animal models.

Disclosures: None



Kevin Prohaska, DO, MPH

Associate Director Office of Clinical Policy
U.S. Food and Drug Administration

Dr. Kevin Prohaska is the Associate Director of the Office of Clinical Policy (OCLiP) within the FDA Office of the Commissioner. His work at OCLiP includes serving as a senior medical policy advisor with a focus on human subjects' protections including issues related to informed consent and bioethics. Dr. Prohaska is a board-certified Family Practice physician with a diverse professional background that includes academic, clinical, military, emergency, and regulatory medicine.

Disclosures: None



Stephan Sanders, BMBS, PhD

Associate Professor of Psychiatry
University of California, San Francisco

Dr. Stephan Sanders is an Associate Professor at the University of California, San Francisco. He trained as a pediatric physician in the UK before pursuing a career in genomics and bioinformatics in the laboratory of Dr. Matthew State at Yale. In 2014, he moved to UCSF to start his own lab in the Department of Psychiatry. Over the last decade, his research has helped characterize the role of rare and de novo genetic variants in the etiology of neurodevelopmental disorders using microarray, exome sequencing, and whole-genome sequencing data. Through the observation and statistical analysis of multiple de novo variants at specific loci in affected individuals, his work has identified numerous Autism Spectrum Disorder (ASD) risk loci, and he has helped apply these approaches to identify over one hundred ASD-associated genes. Dr. Sanders co-leads the whole-genome sequencing working group of the Autism Sequencing Consortium. Extending his research into functional genomics as a member of the PsychENCODE consortium, he has helped identify patterns of gene expression in the developing human brain, genes with sexually dimorphic expression across development, and how rare and common genetic variants impact these patterns. His lab has three main research goals: Identifying genetic risk factors underlying neurodevelopmental disorders; Integrating functional genomic data to interpret the impact of the genetic risk factors; Improving clinical care, including development of genetically-targeted therapies and integration of research data and electronic health records at the point of care.

Disclosures: None

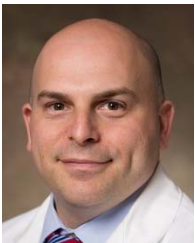


Teresa Sparks, MD, MAS

Assistant Professor, Maternal-Fetal Medicine and Clinical Genetics
University of California, San Francisco

Dr. Teresa Sparks is Assistant Professor of Maternal-Fetal Medicine and Clinical Genetics at the University of California, San Francisco. Her clinical time is spent performing prenatal ultrasounds, developing strategies for genetic evaluations of fetal anomalies, counseling families about the management of fetal anomalies, and caring for pregnant individuals who are diagnosed with a genetic disease themselves. Her research focuses on applying genomic sequencing and fetal phenotyping to discover genetic diseases underlying non-immune hydrops fetalis, a common endpoint of fluid overload in a fetus that can result from a wide range of underlying diagnoses. Her research program integrates cutting edge genomics and fetal imaging to define the unique fetal phenotypes of genetic diseases, achieve an earlier diagnosis, and support unique opportunities for fetal intervention to improve the otherwise grim outcomes associated with non-immune hydrops fetalis.

Disclosures: None



David Stitelman, MD

Assistant Professor of Pediatric Surgery
Yale University

Dr. David Stitelman is a surgeon in the Division of Pediatric Surgery within the Department of Surgery at Yale. He is the surgical Director of the Yale Fetal Care Center. Dr. Stitelman also runs a basic science laboratory with a focus on prenatal therapy to treat genetic and structural diseases before birth.

Disclosures: None



Charlotte Sumner, MD
Professor of Neurology and Neuroscience
Johns Hopkins University School of Medicine

Dr. Charlotte Sumner is a Professor of Neurology and Neuroscience at Johns Hopkins University School of Medicine. Dr. Sumner cares for patients with genetically-mediated neuromuscular diseases and co-directs the Johns Hopkins Muscular Dystrophy Association Care Center, the Spinal Muscular Atrophy (SMA), and the Charcot-Marie-Tooth (CMT) clinics, which deliver multidisciplinary clinical care, engage in international natural history studies, and provide cutting edge therapeutics. Dr. Sumner's laboratory research focuses on the genetic and cellular pathogenesis of motor neuron and peripheral nerve disorders with particular attention to identification of disease genes, characterization of molecular and cellular disease mechanisms, and preclinical development of therapeutics. Her work has been recognized by elected membership in the American Society of Clinical Investigators and the American Association of Physicians. She serves as an advisor to multiple SMA, CMT, and peripheral neuropathy nonprofit foundations, government, and private companies. She is the coeditor of the only comprehensive book on SMA: Spinal Muscular Atrophy Disease Mechanisms and Therapy.

Disclosures: Dr. Sumner has been a paid consultant to Ionis Pharmaceuticals, Biogen, PTC Therapeutics, Roche, Genentech, Avexis, Novartis, Cytokinetics, Sarepta, NuraBio, Atalanta, GenEdit, Epirium, Argenx. She has received sponsored research support from Roche, Biogen, Ionis Pharmaceuticals, and Argenx. She receives book royalties from Elsevier and compensation for her work as an Associate Editor for Journal of Clinical Investigation.



Fyodor Urnov, PhD
Scientific Director, Technology and Translation; Professor of Genetics, Genomics, and
Development
Innovative Genomics Institute, University of California, Berkeley

Dr. Fyodor Urnov is a Professor of Molecular and Cell Biology at the University of California, Berkeley and the Scientific Director of Technology and Translation at the Innovative Genomics Institute (IGI). His research focuses on advancing genome editing technology and pushing the boundaries of how it can be applied to solve real-world problems. Dr. Urnov is a pioneer in the field of genome editing, with a diverse background in academia, industry and the nonprofit sector. He works in collaboration with scientific leadership at IGI to use genome editing to improve human health and well-being. Dr. Urnov's main area of focus is to accelerate the path of CRISPR-based therapies for rare genetic diseases from the lab to the clinic and to develop new CRISPR-based technologies to understand and treat common diseases.

Disclosures: None
