

## **Agenda**

### **Monday 6 March 2023**

08:30 – 09:30 Registration

Refreshments provided

09:30 – 10:00 Introduction and History of Summits

- Linda Partridge, The Royal Society
- Victor Dzau, United States National Academy of Medicine & the United States National Academy of Sciences
- David Baltimore, The California Institute of Technology

Moderator: Robin Lovell-Badge

10:00 – 10:25 Regulation in China following 2018 clinical use of Heritable Human Genome Editing

- Yaojin Peng, Chinese Academy of Sciences
- Joy Zhang, University of Kent

Moderator: Alta Charo

10:25 – 12:50 Hopes and Fears for human genome editing

10:25 – 10:45 Genome editing for precision medicine: status and opportunities:

- David Liu, Broad Institute, Harvard University & Howard Hughes Medical Institute

10:45 – 11:15 *Morning break*

11:15 – 11:45 International equity for access to gene therapies:

- Jantina De Vries, University of Cape Town
- Natacha Salomé Lima, National Scientific and Technical Research Council (CONICET) & Universidad de Buenos Aires
- Ping Yan (in collaboration with Lu Gao, Yang Yang, Wenlong Lu and Wei Zhang), Dalian University of Technology

11:45 – 12:15 Hopes and fears for human genome editing:

- Kelly Ormond, ETH-Zurich & Stanford University
- Filippa Lentzos, Kings College London

12:15 – 12:50 Panel discussion:

- David Liu, Broad Institute, Harvard University & Howard Hughes Medical Institute
- Jantina De Vries, University of Cape Town
- Natacha Salomé Lima, National Scientific and Technical Research Council (CONICET) & Universidad de Buenos Aires
- Ping Yan, Dalian University of Technology
- Kelly Ormond, ETH-Zurich & Stanford University
- Filippa Lentzos, Kings College London

Moderator: Alta Charo

12:50 – 13:50 *Lunch*

During the lunch break a pre-recorded presentation will be played in the main auditorium

CRISPR and Human Genome Editing: Progress and Opportunities

- Jennifer Doudna, Professor at UC Berkeley, Gladstone, HHMI Investigator & Founder and Chair of Innovative Genomics Institute

13:50 – 15:40 Sickle Cell Disease: a case study affecting millions

How to promote innovation while providing the best therapeutic options for patients

Overview and clinical context of Sickle Cell Disease:

- Alexis Thompson, Children's Hospital of Philadelphia and University of Pennsylvania
- Ambroise Wonkam, SickleInAfrica & African Society of Human Genetics

The lived experience of genetic editing treatment for sickle cell disease:

- Victoria Gray, recipient of gene editing treatment for sickle cell

Patient perspectives panel:

- Melissa Creary, University of Michigan
- Arafa Salim Said, Sickle Cell Disease Patients Community of Tanzania
- Gautam Dongre, National Alliance of Sickle Cell Organizations (NASCO) India

A physician/scientist's perspective on developing innovative therapies for a global underserved population:

- Matt Porteus, Stanford University

Moderator: Julie Makani

15:40 – 16:00 *Afternoon break*

16:00 – 17:30 Gene therapy as a benchmark for clinical management of gene-based treatments:

- John Tisdale, National Heart, Lung and Blood Institute, USA National Institute of Health

Therapeutic innovation by gene editing: the growing number of precise engineering options and early clinical findings:

- Dan Bauer, Boston Children's Hospital, Dana-Farber Cancer Institute & Harvard Medical School

Gene editing 2.0: base editing and beyond for sickle cell disease:

- Annarita Miccio, Paris Cite University & Institute Imagine

Bioethicist considerations:

- Kofi Anie, London North West University Healthcare NHS Trust & Imperial College London
- Daima Bukini, Muhimbili University of Health and Allied Sciences

Moderator: Mayana Zatz

## Tuesday 7 March 2023

### 08:30 – 09:30 Role of Civil Society in Setting Research Agenda

Role of patient organisations:

- Mathieu Boudes, Montsouris Consilium
- Bettina Ryll, Melanoma Patient Network Europe & European Commission

Role of activists and DIY community - towards integration of non-establishment research:

- Alex Pearlman, Bioethicist, journalist, and the MIT Media Lab Community Biotechnology Initiative

Role of cultural beliefs:

- Māui Hudson, University of Waikato

Moderator: Cor Oosterwijk

### 09:30 – 10:30 Clinical Research Involving Somatic Editing

Update on the clinical research:

- Amy Wagers, Harvard University

Delivery platforms: streamlining gene targeted therapies:

- Joni L Rutter, National Center for Advancing Translational Sciences, USA  
National Institute of Health

Clinical promise of CAR-T cell therapies using next-generation CRISPR technology:

- Rachel Haurwitz, Caribou Biosciences

Genetic engineering of allogeneic donor cells for acceptance by the host's immune system:

- Sonja Schrepfer, University of California San Francisco & Sana Biotechnology Inc.

Moderator: Luigi Naldini

### 10:30 – 11:00 *Morning break*

### 11:00 – 12:00 Accessibility and price:

- Claire Booth, UCL Great Ormond Street Institute of Child Health
- Mike McCune, Bill & Melinda Gates Foundation
- Steve Pearson, Institute for Clinical and Economic Review

Moderator: Luigi Naldini

### 12:00 – 13:00 *Lunch*

### 13:00 – 15:30 Update of Science and Techniques of genome editing for therapeutic uses and current barriers to clinical applications

Prenatal somatic cell editing for severe early onset genetic disease:

- Tippi Mackenzie, University of California, San Francisco

CRISPR correction of heart and muscle disease:

- Eric Olson, University of Texas Southwestern Medical Center

Gene silencing by epigenome editing for therapeutic uses:

- Angelo Lombardo, San Raffaele - Telethon Institute for Gene Therapy & Vita-Salute San Raffaele University

Creating a turnkey system for engineering CRISPR therapeutics for N=1 genetic disease:

- Fyodor Urnov, University of California, Berkeley

Increasing capacity for diagnosing, manufacturing treatments, and treating genetic diseases:

- Sofonias Kifle Tessema, Africa Centres for Disease Control and Prevention (CDC)

Moderator: George Daley

15:30 – 16:00 *Afternoon break*

16:00 – 17:30 Regulation and Policy Approaches to Increase Accessibility for Somatic Editing

Current known work on somatic editing globally and the relevant regulatory systems in place:

- Katherine Littler, World Health Organization
- Piers Millett, International Biosecurity & Biosafety Initiative for Science

Why in vivo somatic editing must be a priority for low- and middle-income countries:

- Kiran Musunuru, University of Pennsylvania

Ethical considerations for regulation and policy approaches:

- Kaushik Sunder Rajan, University of Chicago

Moderator: Michèle Ramsay

## Wednesday 8 March 2023

9:00 – 10:30 Civil society and human genome editing: roles and challenges in public engagement

- Lluís Montoliu, ARRIGE & National Centre of Biotechnology (CNB-CSIC)
- Sheila Jasanoff, Global Observatory & Harvard University
- Simon Niemeyer, Global Citizens' Assembly on Genome Editing & University of Canberra
- Sarojini Nadimpally, SAMA Resource Group for Women and Health

Are there compelling reasons for heritable human genome editing?

- Ephrat Levy-Lahad, Shaare Zedek Medical Center
- César Palacios-González, University of Oxford
- Tina Rulli, University of California, Davis

Moderator: Françoise Baylis

10:30 – 11:00 *Morning break*

11:00 – 12:40 Update on Science and Techniques for Editing of Gametes

The advantages and disadvantages of editing gametes:

- Amander Clark, University of California, Los Angeles

Update of core technologies:

- Katsuhiko Hayashi, Kyushu University
- Kyle Orwig, University of Pittsburgh
- Mitinori Saitou, Kyoto University

Moderator: Haoyi Wang

12:40 – 13:40 *Lunch*

13:40 – 15:40 Update on Science and Techniques of Editing of Embryos

Chromosomal consequences of gene editing:

- Dieter Egli, Columbia University Irving Medical Center

Analysis of gene editing in human embryos:

- Shoukhrat Mitalipov, Oregon Health & Science University

Genetic approaches to study early lineage specification in human embryos:

- Kathy Niakan, Francis Crick Institute & University of Cambridge
- Dagan Wells, University of Oxford

Monkey embryo editing: perspective on science and technology:

- Yuyu Niu, State Key Laboratory of Primate Biomedical Research & Kunming University of Science and Technology

Moderator: Robin Lovell-Badge

15:40 – 16:10 *Afternoon break*

16:10 – 17:00 How to enforce research policies and ethical principles for human genome editing

- María de Jesús Medina Arellano, National Autonomous University of Mexico
- Yaojin Peng, Chinese Academy of Sciences
- Leigh Turner, University of California, Irvine

Moderator: Māui Hudson

17:00 – 18:00 Concluding Remarks from Organising Committee

Moderator: Robin Lovell-Badge

## **Organising Committee Biographies**

### **Robin Lovell-Badge (Chair)**



Principal Group Leader, Laboratory of Stem Cell Biology and Developmental Genetics, The Francis Crick Institute.

Robin Lovell-Badge, CBE, FRS, FMedSci, obtained his PhD at University College London in 1978 and was a postdoc in Cambridge, both with Martin Evans. After an EMBO fellowship in Paris he established his independent laboratory in 1982 at the MRC Mammalian Development Unit, UCL, directed by Anne McLaren. In 1988 he moved to the MRC National Institute for Medical Research, becoming Head of the Division of Stem Cell Biology and Developmental Genetics in 1993. The NIMR was incorporated into the Francis Crick Institute in April 2015. In 1990, his lab

discovered Sry, the Y-linked sex determining gene and the first members of the Sox gene family. He has had long-standing interests in the biology of stem cells, in how genes work in the context of embryo development, and how decisions of cell fate are made. Major themes of his current work include sex determination, development of the nervous system and pituitary, and the biology of stem cells within these systems. He is also very active in both public engagement and policy work, notably around stem cells, genetics, human embryo and animal research, and in ways science is regulated and disseminated. He chairs the Royal Society's Genetic Technologies Programme and has been involved in the human genome editing Summit meetings since their inception. He was elected a member of EMBO (1993), a Fellow of the Academy of Medical Sciences (1999), the Royal Society (2001), the Royal Society of Biology (2011), and the American Association for the Advancement of Science (AAAS) (2018). He has received the Louis Jeantet Prize for Medicine (1995), the Amory Prize (1996), the Feldberg Foundation Prize (2008), the Waddington Medal of the British Society for Developmental Biology (2010), the ISSCR Public Service Award (2021), and the Genetics Society Medal (2022). He was awarded a CBE in the 2018 New Year's Honours List. He is an honorary professor at University College, London and at King's College, London, and a Special Visiting Professor at the University of Hong Kong.

### **David Baltimore**



David Baltimore is President Emeritus and Judge Shirley Hufstедler Professor of Biology at Caltech. Awarded the Nobel Prize in 1975 for research in virology, Baltimore has profoundly influenced national science policy on such issues as recombinant DNA research and the AIDS epidemic.

Dr Baltimore graduated from Swarthmore College with a degree in chemistry. He took graduate courses at Massachusetts Institute of Technology and received his PhD from Rockefeller University. He was a postdoctoral fellow at MIT and Albert Einstein College of Medicine. He was

a Research Associate at The Salk Institute in 1965 and joined the faculty at MIT in 1968. He has served as Director of the Whitehead Institute for Biomedical Research, President of Rockefeller University, and President of Caltech.

He was awarded the 1999 National Medal of Science, 2000 Warren Alpert Foundation Prize and 2021 Lasker-Koshland Special Achievement Award in Medical Science. He is a member of the National Academy of Sciences, fellow of the American Academy of Arts and Sciences, and a foreign member of the Royal Society of London and the French Academy of Sciences. He has been President and Chair of the American Association of the Advancement of Science.

## **Françoise Baylis**



Françoise Baylis CM, ONS, PhD, FRSC, FCAHS, FISC is Distinguished Research Professor Emerita, Dalhousie University, Canada. She is a philosopher whose innovative work in bioethics, at the intersection of policy and practice, has stretched the boundaries of the field. Her work challenges us to think broadly and deeply about the direction of health, science and biotechnology. It aims to move the limits of mainstream bioethics and develop more effective ways to understand and tackle public policy challenges.

Baylis is the author of *Altered Inheritance: CRISPR and the Ethics of Human Genome Editing*. Her book won the 2020 PROSE Award in

Clinical Medicine. Natalie de Souza writes in *The New York Review of Books* "She offers an authoritative, comprehensive guide to the ethical issues around CRISPR, and her central message is clear: heritable human genome editing shouldn't be treated as inevitable, and the decision to undertake it should be a collective one." Adam Hayden writes in *Science* "Commitments to justice, responsibility, accountability, and consensus building are features of a socially just science and bioethics. Toward this end, *Altered Inheritance* is a foundational tool in the path ahead."

Baylis was a member of the Organising Committee for the First and Third International Summit on Gene Editing (2015 and 2023), a member of the WHO Expert Advisory Committee on Developing Global Standards for Governance and Oversight of Human Genome Editing (2019-2021), and a member of WHO Working Groups on a Global Guidance Framework for the Responsible Use of the Life Sciences (2021). She is a member of the Governing Board of the International Science Council and Vice-Chair of its Committee for Freedom and Responsibility in Science.

Baylis is a member of the Order of Canada and the Order of Nova Scotia, as well as an elected Fellow of the Royal Society of Canada, the Canadian Academy of Health Sciences, and the International Science Council. In 2022, she was awarded the Killam Prize for the Humanities, Canada's most distinguished award for humanities scholars.

## **Ewan Birney**



Ewan Birney is Deputy Director General of EMBL. He is also Director of EMBL-EBI with Dr Rolf Apweiler and runs a small research group.

Ewan completed his PhD at the Wellcome Sanger Institute with Richard Durbin. In 2000, he became Head of Nucleotide data at EMBL-EBI and in 2012 he took on the role of Associate Director at the institute. He became Director of EMBL-EBI in 2015. In 2020, Ewan became the Deputy Director General of EMBL. In this role, he assists the EMBL Director General in relation to engagement with EMBL Member States and external representation.

Ewan led the analysis of the Human Genome gene set, mouse and chicken genomes and the ENCODE project, focusing on non-coding elements of the human genome. Ewan's main areas of research include functional genomics, DNA algorithms, statistical methods to analyse genomic information (in particular information associated with individual differences in humans and Medaka fish) and use of images for chromatin structure.

Ewan is a non-executive Director of Genomics England, and a consultant and advisor to a number of companies, including Oxford Nanopore Technologies, Dovetail Genomics and GSK. Ewan was elected an EMBO member in 2012, a Fellow of the Royal Society in 2014 and a Fellow of the Academy of Medical Sciences in 2015. In 2019, Ewan became a Board Member of the Biotechnology and Biological Sciences Research Council (BBSRC).



He has received a number of awards including the 2003 Francis Crick Award from the Royal Society, the 2005 Overton Prize from the International Society for Computational Biology and the 2005 Benjamin Franklin Award for contributions in Open Source Bioinformatics.

### **Alta Charo**



R. Alta Charo, J.D., is the Warren P. Knowles professor emerita of law and bioethics at the University of Wisconsin–Madison (UW) and since 2020 works as an independent consultant to industry and government on biotechnology ethics, regulation and policy. She also serves as co-chair of the safety, security, sustainability and social responsibility unit of BioMADE, a US-based consortium of biotechnology companies and academic centers. Prior to her arrival at UW in 1989, Ms. Charo served as associate director of the Legislative Drafting Research Fund of Columbia University, Fulbright lecturer in American law at the Sorbonne in Paris, legal analyst at the congressional Office of Technology

Assessment, and American Association for the Advancement of Science (AAAS) diplomacy fellow at the US Agency for International Development. Also, in government, she was a member of President Clinton's National Bioethics Advisory Commission and a senior policy advisor in the FDA's Office of the Commissioner under the Obama administration. At the US National Academies of Sciences, Engineering, and Medicine, Ms. Charo has served on numerous committees, the committee that issued the 2004 report 'Biotechnology Research in the Age of Terrorism'; the committee on emerging science, technology and innovation; and the committees on emerging infectious diseases and COVID-19 vaccine and monoclonal antibody allocation frameworks. She co-chaired the National Academies committee that wrote guidelines for embryonic stem cell research and the committee that developed recommendations for U.S. policy and global principles for human genome editing research and clinical trials. Ms. Charo recently completed service on the World Health Organization's committee on global governance of genome editing. She is an elected member of the US National Academy of Medicine and an elected Fellow of the AAAS and of the American Academy of Arts and Sciences.

### **George Daley**



George Q. Daley, MD, PhD is Dean and Caroline Shields Walker Professor of Medicine at Harvard Medical School. As a physician-scientist his research has focused on stem cell and cancer biology, with an emphasis on hematopoietic development and diseases of the bone marrow, blood and immune system. On behalf of the US National Academies of Science, Engineering and Medicine he has served on the organizing committee for the First and Second International Summits on Human Genome Editing and serves as co-chair of the National Academy of Medicine Committee on Emerging Science, Technology and Innovation. On behalf of the International Society for Stem Cell

Research, he has co-authored international guidelines for the conduct and clinical translation of stem cell research and regenerative medicine (2006, 2008, 2016, 2021). Daley earned his AB and MD degrees from Harvard and a PhD in biology from MIT. He has been a trainee, fellow and staff physician at the Massachusetts General Hospital, Brigham and Women's Hospital, Dana Farber Cancer Institute and Boston Children's Hospital. Prior to becoming Dean at HMS, he was an investigator of the Howard Hughes Medical Institute and Director of the Pediatric Stem Cell Transplantation Program of the Dana Farber Cancer Institute and Boston Children's Hospital.

### **Javier Guzman**



Javier Guzman is the Director of Global Health Policy and a Senior Fellow at the Center for Global Development. Previously, he was the Technical Director of the USAID-funded Medicines, Technologies, and Pharmaceutical Services (MTaPS) Program at Management Sciences for Health (MSH), helping over 20 governments in Africa and Asia build their health system to ensure sustainable access to and appropriate use of safe, effective, quality-assured, and affordable medicines and pharmaceutical services. Between 2013 and 2018, he played different roles within the Colombian government, first as Deputy Director of the health technology assessment agency, then as Director of Medicines and Health Technologies at the Ministry of Health and finally, as Director General of the Colombian Food and Drug Surveillance Institute, one of the eight National Regulatory Authorities of regional reference in the Americas.

Javier has worked as a physician, researcher, analyst and policy maker in the United States, the United Kingdom, Australia, and his native Colombia. He is interested in how global health challenges such as Covid, access to medicines and other health technologies and antimicrobial resistance, can offer a window of opportunity to improve the efficiency and effectiveness of global institutions and build more sustainable and equitable health systems.

As Director of Research for global health nongovernmental organizations in the United Kingdom and Australia, he managed portfolios of health consulting and research projects focusing on pharmaceutical policy, pharmaceutical systems strengthening, and diseases that disproportionately affect low- and middle-income countries.

In addition to his medical degree, Javier has an MSc in Health Policy, Planning and Financing from the London School of Economics and Political Science and the London School of Hygiene and Tropical Medicine and an MBA (Executive) from the Australian Graduate School of Management.

### **Daria Julkowska**



Daria Julkowska has over 15 years of experience in research and management. She is the Scientific Coordinator of the European Joint Programme on Rare Diseases that brings together different type of stakeholders (researchers, funders, clinicians & patients) from 35 countries from Europe and beyond, and also is responsible for the coordination of the IRDiRC Scientific Secretariat. This position allows her to implement the strategic rare disease research and funding recommendations of IRDiRC to the development of EJP RD which includes the participation of the European Research Networks. She is involved in the rare diseases field since 2010, starting from E-Rare, the

ERA-Net for Research programmes on rare diseases, where for the first two years she occupied the position of the project manager to finally (April 2013-December 2018) take over the coordination of the programme. As the coordinator, she developed and put into action a set of collaborations facilitating rare diseases research, including the partnerships with European Research Infrastructures and Patients' Organizations. She has an extensive knowledge and understanding of European funding schemes and programmes. Dr Julkowska obtained her international PhD in molecular biology at the University of Paris XI, France and University of Gdansk, Poland in 2005. She pursued her scientific vocation by the post-doctoral experience in cellular biology, at Institut Pasteur, Paris and extensive training in communication and European Union counselling. She also holds MSc in Management of Research from the University of Paris Dauphine.

### **Julie Makani**



Professor Julie Makani is a Physician Scientist in the Department of Haematology and Blood Transfusion at Muhimbili University of Health and Allied Sciences ([MUHAS](#)), where Tanzania established one of the world's largest single-center study cohorts for sickle cell disease. She serves as Principal Investigator of the Sickle Cell programme in MUHAS and Sickle Pan African Research Consortium (SPARCO) Clinical Coordinating Centre (CCC) within [SickleInAfrica](#) network. Site Principal Investigator for MUHAS for [H3ABioNet](#) and Co- Principal Investigator [SickleGenAfrica](#). These networks are strengthening health, education, advocacy and research in sickle cell disease and with related diseases and sectors. The partnerships are at different levels: institutional, national, regional, Africa and Global. Through this work, there is progress in establishment of platforms for health (Centres of Excellence; Blood Transfusion Programmes) and genomic research (H3Africa, SickleGenAfrica). These platforms are available for gene therapy research and are potential opportunities for strengthening specialized health services and introducing curative therapies within health systems in countries in Africa in an equitable manner. The aim is to use sickle cell disease as a model to establish scientific and healthcare solutions in Africa that are locally relevant and globally significant.

Professor Makani (MD, PhD, FRCP, FTAAS) trained in Medicine (Tanzania), specialized in Internal Medicine (UK), and completed her PhD in clinical epidemiology of sickle cell disease (UK). She has received numerous national and international awards for her academic and scientific achievements. She was a Wellcome Trust Research Fellow [2003 – 2009; 2012 - 2017], [Tutu Leadership Fellow](#) (2009) and Honorary Visiting Research Fellow, University of Oxford (2003 - 2016). She received the 2011 [Royal Society Africa Award](#) on the application of genomic research to improve health and find a cure for sickle cell disease. She is a Fellow of the Royal College of Physicians of UK and Fellow of the Tanzania Academy of Sciences.

### **Christopher McCabe**



Dr Christopher McCabe is Professor of Health Economics at Queens University Belfast, jointly appointed to the Faculty of Medicine, Health and Life Sciences and Queens Management School. He was previously the CEO and Executive Director of the Institute of Health Economics, a not-for-profit research institute in Alberta Canada.

Dr McCabe has 30 years' experience as a university based health economist. He trained and worked for 20 years in the UK before emigrating to Canada. His PhD was concerned with the methods for valuing paediatric health. During his time in the UK, he held Full Professorships at the Universities of Sheffield, Warwick and Leeds. In the United Kingdom he was the founding Director of both the NICE Decision Support Unit, and the Academic Unit of Health Economics at the University of Leeds. He was more recently a Professor of Health Economics at the University of Alberta, where he was appointed to the Capital Health Endowed Research Chair. In this post he led two Genome Canada funded research groups focused on the evaluation, adoption and implementation of Precision Medicine technologies, as well as contributing to Stem Cell Network funded research on the Ethical, Legal and Policy Issues in regenerative medicine.

Dr McCabe has contributed to a number of standard setting documents related to the evaluation of innovative health technologies including the second edition of the NICE Guide to the Methods of Health Technology Appraisal, the fourth edition of the CADTH Guidelines for the Economic Evaluation of Health Technologies, and third edition of the ISSC Guidelines for the translation of stem cell research and regenerative medicine. He was lead author of the 2018 CADTH Guideline on the economic evaluation of co-dependent therapies. He has contributed key papers to the literature the reimbursement of treatments for rare diseases. More recently D. McCabe advised the Patented

Medicines Price Review Board on the technical issues related to the revision of their regulations for setting the price of patented drugs in Canada. He is currently Chair of the Royal Society of Canada COVID Task Force Working Group on the Economy, and a member of the same task force's Working Group on Health Care post-COVID.

### **Luigi Naldini**



Luigi Naldini is the Director of the San Raffaele Telethon Institute for Gene Therapy and professor at the San Raffaele University in Milan, Italy. For the past 25 years, he has pioneered the development and applications of lentiviral vectors for gene transfer, which have become one of the most widely used tools in biomedical research and, upon recently entering clinical testing, are providing a long-sought effective treatment for several otherwise deadly human diseases. His work also contributed to advance the use of artificial nucleases for targeted genome editing in cell and gene therapy. Luigi received his medical degree from the University of Torino, Italy, and his PhD from the University of Rome. He is Member of the European Molecular Biology Organization (EMBO) and has been President of the European Society of Gene and Cell Therapy (ESGCT). He was awarded the Outstanding Achievement Award from the American Society of Gene and Cell Therapy (ASGCT) and from ESGCT, the Jimenez Diaz Prize, the Beutler Prize from the American Society of Hematology (ASH) and the Jeantet-Collen Prize for Translational Medicine. He was nominated "Grande Ufficiale dell'Ordine Al Merito della Repubblica Italiana", one of the highest-ranking honour in Italy.

### **Cor Oosterwijk**



Cor Oosterwijk, PhD is the Director of VSOP - the Patient Organisation for Rare and Genetic Diseases in The Netherlands. VSOP is a national umbrella organisation with a membership of about 100 patient organisations, and is partner of EURORDIS – Rare Diseases Europe. VSOP's staff is working on reproductive health, timely diagnosis, genetic screening, quality of care, medical ethics and (access to) therapeutic innovation.

Cor is a patient representative since 2001, and as such represented in several (inter)national committees, projects and organisations in the field of health policy, rare diseases and clinical genetics. At national level, this includes governmental committees in the field of reproductive health - preimplantation genetic testing, prenatal and neonatal screening - as well as his membership of the Insured Package Advisory Committee of the Dutch Healthcare Institute. At international level, he is a board member of EGAN – the European Patients Network for Medical Research and Health; and founder and chairman of the Preparing for Life Foundation, that worked with WHO on international recommendations for preconception care.

Oosterwijk is a medical biologist by education (Utrecht University, The Netherlands). Thereafter, he taught agricultural biotechnology and gene editing for two years. Subsequently, he started his scientific work on Islet Amyloid PolyPeptide overproduction in type 2 diabetes mellitus, studied in tumour models and transgenic mice. Before he started as a patient advocate, he worked for five years as an international clinical trial manager cardiovascular.



### **Lily Paemka**



Dr Lily Paemka is Geneticist, Principal Investigator, Senior lecturer, and the coordinator of the Genetics Programme at the Biochemistry, Cell and Molecular Biology Department, University of Ghana (UG), West Africa. She serves on the executive committee of the GhGenome Project and is the Director of Human Genetics at Yemaachi Biotechnology Ltd, Ghana.

She earned her Bachelor of Science and MPhil degrees at the University of Ghana and then received her doctorate in Genetics at the University of Iowa, USA.

Dr Paemka characterized genes implicated in autism spectrum disorders and epilepsy for her doctoral studies. As a postdoctoral fellow, she elucidated the effect of loss of the *CFTR* gene on myeloid and epithelial cells in cystic fibrosis pathogenesis. This was followed by a postdoctoral stint in cancer genetics at the University of Iowa, where she helped describe the role of the tumour suppressor gene *TFAP2A* in cancer. She won the DELTAS (Developing Excellence in Leadership, Training and Science African postdoctoral research grant and relocated to the University of Ghana in 2017 to characterize breast cancer genetic risk factors in Ghanaian Women at the West African Centre for Cell Biology of Infectious Pathogens (UG).

The Paemka lab aims to provide comprehensive genetic data underpinning cancers and other genetic conditions in Blacks and develop resources that could lead to novel molecular therapeutic options. This is a bid to rectify the vast disparity in genetic studies between high income countries and sub-Saharan Africa. Her current research involves characterising genetic and pathogenic breast cancer risk factors in Ghanaian women, prostate cancer epigenomics and multiple myeloma cytogenetics and genomics. She is also involved in COVID-19 research where she is elucidating the role of the human genome in mediating human response to SARS-Cov-2 infection in Ghanaians.

To mitigate the high rates of late presentation in Ghanaian women, the Paemka lab in 2018 established and spearheaded the annual Nufu festival to raise breast cancer awareness and provide free breast examinations to ameliorate the disproportionately high mortality rate associated with the disease in Ghanaian women.

She is currently a recipient of the UNESCO Organization of Women in Science for the Developing World (OWSD) fellowship.

### **Michèle Ramsay**



Michèle Ramsay (PhD) is director of the Wits Sydney Brenner Institute for Molecular Bioscience (SBIMB), Professor in Human Genetics and South African Research Chair in Genomics and Bioinformatics of African Populations. Her research aims to shed light on the role of African population genomic variation in susceptibility to diseases, in the context of the ethnic and environmental diversity across the continent. In 2019/2020 she was a member of the International Commission on the Clinical Use of Human Germline Genome Editing (convened by the UK Royal Academy of Science and U.S. National Academy of Medicine) (published in September 2020 (<https://www.nap.edu/catalog/25665>)). She

is on the Editorial Board for Human Molecular Genetics and is Guest Editor for a Special Issue on “Evolutionary Genetics in Africa”. She is on the Scientific Advisory Committee of the H3Africa Bioinformatics Network (H3ABioNet), Advisory Panel (Africa) for the Wellcome Genome Campus Advanced Courses and Scientific Conferences Overseas Courses, External Advisory Board of EU-Africa PerMed Consortium, Omics lead for the formative phase of the African Population Cohorts Consortium (APCC), and co-chair of the International Hundred-thousand+ Cohorts Consortium (IHCC). Michèle is President of the International Federation of Human Genetics Societies and chair of the International Scientific Programme Committee for the International Congress of Human Genetics. Her recent awards include: DSI Distinguished Woman Researcher 2019 award (August 2019); NSTF-

South32 Lifetime Achievement Award (July 2020); and Gold Medal from the South African Medical Research Council (March 2021); Science Team Award to the SBIMB from the National Research Foundation (South Africa)(2022). Ramsay has supervised and mentored young scientists at Wits and across Africa and continues to promote capacity development for genetic and genomic research and genetic services in African settings.

### **Elisa Reis**



Elisa holds a Ph.D. in Political Science from the Massachusetts Institute of Technology, a master's degree in Political Science from the Rio de Janeiro. Her publications focus mainly on social theory, inequalities, elite perceptions of poverty and inequality, and current global transformations.

Elisa has extensive experience in research and teaching, as well as participating in various scientific organizations and advisory boards. As part of the International Social Science Council (ISSC) Executive Committee, and later as Vice-President for scientific activities of the same Council, Elisa was involved in several scientific initiatives. In 2018, when the ISSC and International Science Council (ICSU) joined together to form the International Science Council (ICS), Elisa was elected Vice-President of the new organization for the period 2018-2021, and is currently a Fellow of ISC. She is an active member of the Brazilian Academy of Sciences, and of The World Academy of Sciences (TWAS).

### **Haoyi Wang**



Haoyi Wang, Ph.D., leads a research group in the State Key Laboratory of Stem Cell and Reproductive Biology at the Chinese Academy of Sciences' Institute of Zoology. The Wang laboratory focuses on developing novel technologies to achieve efficient and specific genome engineering and applying them to study the function of genes and establish novel therapeutic methods. His laboratory has developed a zygote electroporation of nuclease method to generate genetically modified mouse models with high throughput and efficiency, the Casilio method to regulate gene transcription, and a method to generate CAR-T cells with multiplex gene editing. Dr. Wang previously worked on the

development of a variety of genome engineering technologies, including a transposon-based "calling card" method for determining the genome-wide binding locations of transcription factors, TALEN-mediated genome editing in human pluripotent stem cells and mice, CRISPR-mediated multiplexed genome editing in mice, and CRISPR-mediated gene activation in human cells. Dr. Wang received his Ph.D. from Washington University in St. Louis.

### **Mayana Zatz**



Mayana Zatz holds a PhD in Human and Medical Genetics. She is professor of Genetics at the University of S.Paulo and the Director of the Human Genome and stem-cell center, which has 3 aims: basic research, technology transfer and education. The Centre has been visited by more than 100,000 patients and relatives with genetic disorders for genetic counseling by a multidisciplinary team (including diagnosis, genetic testing in at-risk relatives, management orientation and follow-up) for two main purposes: patients contributing to new researches and new findings helping patients. Mayana's research focus has been neuromuscular disorders and aging. More recently she has been involved with the

oncolytic properties of zika virus (transforming our enemy in an ally against brain tumors) and

currently also coronavirus pandemic (aiming to identify genetic variants associated to resistance against Sars-Cov-2).

She published over 400 peer-reviewed papers which were cited more than 22.500 times. In 2001 she was awarded the L'Oréal/Unesco prize for "Women in Sciences" representing Latin America and in 2004 the TWAS Prize in Basic Medical Sciences (Academy of Sciences for Developing countries )

Most importantly, since the beginning of Mayana's career, she has been involved in genetic counseling and ethical issues related to scientific advances. In 2005 in the Brazilian Congress Mayana defended the approval of a Bill allowing research with embryonic stem cells which was definitively approved by the Supreme Court in 2008. The Zatz group is using CRISPR technology in cell lines derived from patients with different neuromuscular disorders. She has been faced with many questions raised by the patients' families regarding the clinical use of CRISPR technology for treating neuromuscular disorders. Mayana published a book in 2011 (not translated to English) with the title: Gen ETHICS: choices our grandparents did not have. This book, intended for lay people, has been adopted by many schools and discussed with young students. Mayana believes that the possibility of gene editing opens new highly relevant questions that need to be discussed with society and she is very honored to participate in these discussions.

## **Speaker Biographies**

### **Kofi Anie**



Dr Kofi Anie MBE is the Psychology Service Lead for children and adults with sickle cell disease and thalassaemia at London North West University Healthcare NHS Trust, and the Deputy Clinical Director of the West London Haemoglobinopathies Coordinating Centre.

Dr Anie is a Fellow of the Royal Society of Medicine and Associate Fellow of the British Psychological Society. He has been involved in development of national clinical guidelines and standards of care for sickle cell disease and thalassaemia including the National Institute for Health and Care Excellence (NICE) Guideline – “Sickle Cell Acute Pain Episode,” He is a Scientific Advisor to the Sickle Cell Society, and a Haemoglobinopathies Editor for the Cochrane Library.

### **Daniel E. Bauer**



Daniel Bauer is a physician-scientist whose research focuses on functional genomics to dissect determinants of blood cell development and disease and develop innovative therapies. He identified therapeutic genome editing of the *BCL11A* erythroid enhancer as a favorable strategy for the  $\beta$ -hemoglobinopathies, now validated in several clinical trials, as well as identified a therapeutic gene editing strategy for amelioration of ELANE-mutant severe congenital neutropenia, described a core NuRD subcomplex critical for fetal hemoglobin (HbF) repression and uncovered ZNF410 as a transcription factor that singularly regulates CHD4 and thus silences HbF. His clinical work focuses on non-malignant hematology. He is Director of the Gene Therapy Program at Dana-Farber/Boston Children's Cancer and Blood Disorders Center and Fredrickson Associate Professor of Pediatrics at Harvard Medical School. He was recipient of the Young Investigator Award (Society for Pediatric Research 2017), Outstanding New Investigator Award (American Society of Gene and Cell Therapy 2020), and elected member of the American Society of Clinical Investigation (2021).

### **Claire Booth**



Professor Claire Booth, MBBS PhD is a Gene Therapist and Paediatric Immunologist at UCL Great Ormond Street Hospital Institute of Child Health in London and leads the clinical stem cell gene therapy programme. She was appointed as a Consultant in Paediatric Immunology at Great Ormond Street Hospital in 2014 and holds an honorary position at Boston Children's Hospital/Dana Farber Cancer Institute and Harvard Medical School.

Claire now works as a clinical academic leading an expanding number of gene therapy clinical trials at Great Ormond Street Hospital which treats patients with immune deficiencies, haematological and metabolic disorders. She is also the co-founder of the AGORA initiative (Access to Gene therapies fOr RAre disease) which has founding members across 6 European countries and brings together clinicians and scientist with direct experience of developing and delivering ex vivo gene therapies for rare diseases, aiming to facilitate access to effective gene therapies for treatment of patients with ultra-rare diseases.



### **Mathieu Boudes**



Mathieu Boudes is a patient engagement expert providing strategic advice, stakeholder engagement, implementation support, and partnership services to the pharmaceutical companies, patient advocacy groups and larger consultancies to unlock patient insights to R&D, clinical and access teams and public-private consortia.

### **Daima Bukini**



Dr. Daima Bukini has been working at Muhimbili University of Health and Allied Sciences (MUHAS) in Tanzania for the past 16 years. She holds a BSc in Microbiology (University of Dar es Salaam), Masters Degree in Bioethics (University of Pennsylvania) and Masters in Public Health (MUHAS). She completed her PhD in 2022, investigating the Ethical, Legal and Sociocultural Issues (ELSI) on Newborn Screening (NBS) for Sickle Cell Disease (SCD) in Tanzania. She is also a chair of the ELSI working group for the Sickle in Africa, which forms the Sickle Pan African Research Consortium (SPARCO) and Sickle

Africa Data Coordinating Centre (SADaCC). Dr. Bukini is also a co- principal investigator of the Sickle CHARTA project at MUHAS aiming to strengthen SCD advanced therapy initiatives across four main areas; Health, Advocacy, Research and Training.

### **Amander Clark**



Professor Amander Clark PhD is a stem cell scientist, geneticist and developmental biologist who is internationally recognized for her work on the germline and in vitro gametogenesis. Professor Clark has authored over one hundred research articles, and is regularly invited to speak on the use of stem cells to understand fertility and the disease of infertility. Professor Clark's research is currently supported by grants from the National Institute of Health and the Bill and Melinda Gates Foundation. In 2022 she was awarded the Founder's Medal from the Australian and New Zealand Society for Reproductive

Biology. Professor Clark was recruited to UCLA in 2006, awarded tenure in 2012, promoted to full Professor in 2015, and in 2017 became Department Chair of the UCLA Department of Molecular Cell and Developmental Biology. Professor Clark is currently the inaugural Director of the UCLA Center for Reproductive Science, Health and Education, and President-Elect of the International Society for Stem Cell Research, a global non-profit that promotes excellence in stem cell science and applications to human health

### **Melissa Creary**



Melissa Creary, PhD, MPH is an Assistant Professor in the Department of Health Management and Policy in the School of Public Health. She is also Senior Advisor of Public Health, Policy, and Equity at the American Thrombosis and Hemostasis Network (ATHN) and Associate Director of Anti-Racism for Michigan Social Health Interventions to Eliminate Disparities (MSHIELD) at Michigan Medicine. Over a nine-year career at the Centers for Disease Control and Prevention in the Division of Blood Disorders, she helped create and lead the first national program and data collection system for sickle cell disease

(SCD) at the agency.

Dr. Creary is an interdisciplinary social scientist who has worked with the sickle cell and bleeding disorder community as a scientist, policy maker, and public health researcher for over 20 years. Her primary research interests include how science, culture, and policy intersect, particularly around ethical, legal, and social concerns (ELSI). Dr. Creary is also living with sickle cell disease and has been invested in the increased care and quality of life for all persons living with SCD.

### **María de Jesús Medina-Arellano**



María de Jesús Medina-Arellano is a PhD in Bioethics and Medical Jurisprudence. She is also a qualified lawyer who graduated from the Autonomous University of Nayarit (UAN), Mexico in 2004. Currently, she is a permanent research professor at the Legal Research Institute in the National Autonomous University of Mexico (UNAM). She is a member of the National Research System in Mexico, SNI- level 2, in Mexico's CONACyT. Currently, she is on annual sabbatical research as an academic visiting scholar at the Uehiro Centre for Practical Ethics at the University of Oxford and the Centre for Social Ethics and Policy at the Department of Law of the University of Manchester in the UK.

She is a member of Mexican Society for Stem Cell Research since 2016. In 2019 she obtained the medal of women excellence in science at UNAM, Sor Juana Inés de la Cruz, and in 2020 she was granted with the price National University for Youth Scientists in Social Sciences at UNAM.

### **Jantina Devries**



Jantina de Vries is the director of The Ethics Lab at the Neuroscience Institute and an Associate Professor in Bioethics in the Department of Medicine of the University of Cape Town. Her expertise is in the ethics of African genomics research, and she is currently leading a large research and teaching program that brings together knowledge from the African humanities with the ethics of new and emerging health technologies. She was a member of the WHO Genome Editing Expert Advisory Committee as of the H3Africa Steering Committee. She is a member of: the Research Ethics Board of Médecins Sans Frontières, the Steering Committee of the Global Forum for Bioethics in Research and the Advisory Board for the Center for ELSI Resources and Analysis (CERA) at Stanford University.

### **Gautam Dongre**



Gautam Dongre is the father of two Sickle Cell Disease (SCD) warriors. As Secretary of National Alliance of Sickle Cell Organizations (NASCO), India's 1st national patient advocacy group for SCD, he represents the Government of India (GOI) National SCD Council, India's highest policymaking multi-stakeholder forum on SCD. He is a Board Member of Global Alliance of Sickle Cell Disease Organization GASCO. Gautam has been working with Sickle Cell Society of India, Maharashtra for a decade and is passionate about preventing new births of Sickle Cell across India.

### **Dieter Egli**



Dieter Egli is the Maimonides Assistant Professor of Cell and Developmental Biology, Department of Pediatrics, Naomi Berrie Diabetes Center, Columbia Stem Cell Initiative, and Department of Obstetrics and Gynecology, Columbia University. Dr. Egli received his Ph.D. in molecular biology in 2003 from the University of Zurich, Switzerland, with the mentorship of Prof. Walter Schaffner. He then joined the laboratory of Prof. Kevin Eggan at Harvard University as a postdoctoral fellow where he studied somatic cell reprogramming. Joining the New York Stem Cell Foundation Research Institute as one of the founding

members in 2008, first as a postdoctoral fellow and then as an independent group leader, he worked on understanding diabetes using pluripotent stem cells, mitochondrial replacement and somatic cell nuclear transfer. In 2014, he started his laboratory at Columbia University Medical Center, performing studies on genome stability in the contexts of gene editing, early embryonic development, somatic cell reprogramming, and cell differentiation.

### **Victoria Gray**



Victoria Gray is a 37-year-old wife and mother of 4. She has battled with sickle cell disease type ss for 34 years. Dealing with unbearable pain, multiple blood transfusions, countless hospital visits and energy draining pain medications almost made life unbearable. In 2018 she made the life changing decision to try gene therapy. It was one of the best decisions she has ever made. Victoria hopes that her story will reignite the torch of hope inside the lives of many others whose light has begun to dim like hers once did.

### **Rachel Haurwitz**



Rachel is a co-founder of Caribou Biosciences and has been its President and Chief Executive Officer and a director since the company's inception in 2011. Caribou Biosciences is a clinical-stage CRISPR genome-editing biopharmaceutical company dedicated to developing allogeneic CAR-T and CAR-NK cell therapies for hematologic malignancies and solid tumors. The company's genome-editing platform, including its proprietary Cas12a CRISPR hybrid RNA-DNA (chRDNA) technology, enables superior precision to develop cell therapies that are armored to provide durable antitumor activity. Rachel is an inventor on patents and patent applications covering multiple CRISPR-based technologies and has co-authored several scientific papers in high-impact journals characterizing CRISPR-Cas systems. Rachel earned an A.B. in Biological Sciences from Harvard College and a Ph.D. in Molecular and Cell Biology from the University of California, Berkeley.

### **Katsuhiko Hayashi**



Katsuhiko Hayashi is a professor of Genome Biology, Graduate School of Medicine, Osaka University, working on germ cell development and its reconstitution in vitro through his career: 1994-1996, MS course of Meiji University; 1996-2002, an assistant professor in Tokyo University of Science; 2002-2005, a staff researcher in Osaka Medical Center (Ph.D. 2004); 2005-2009, post-doctoral fellow in the Gurdon Institute, University of Cambridge; 2009-2014, associate professor in Kyoto University; and 2014-2021 a full professor in Kyushu University. He has been in his current position in Osaka University since 2021.

### **Māui Hudson**



Māui Hudson affiliates to the Whakatōhea Nation in Aotearoa New Zealand and is a member of the Whakatohea Maori Trust Board. He is an Associate Professor and Director of Te Kotahi Research Institute at the University of Waikato. He is a founding member of Te Mana Raraunga Maori Data Sovereignty Network and Global Indigenous Data Alliance and is a co-author of the CARE Principles for Indigenous Data Governance and the Te Mata Ira Guidelines for Genomic Research with Maori.

### **Sheila Jasanoff**



Sheila Jasanoff is Pforzheimer Professor of Science and Technology Studies at the Harvard Kennedy School. A pioneer in the social sciences, she explores the role of science and technology in the law, politics, and policy of modern democracies. She founded and directs the STS Program at Harvard, where she also formed the Science and Democracy Network; previously, she was founding chair of the STS Department at Cornell. In 2022, she received the Government of Norway's Holberg Prize for law, humanities, and social sciences. Her other honors include the SSRC's Hirschman prize, the Humboldt Foundation's Reimar-Lüst award, and a Guggenheim Fellowship. She is a member of the American Academy of Arts and Sciences and the American Philosophical Society, foreign member of the British Academy and the Royal Danish Academy, and member of the Council on Foreign Relations. She holds AB, JD, and PhD degrees from Harvard, and honorary doctorates from the Universities of Twente and Liège.

### **Joni L. Rutter**



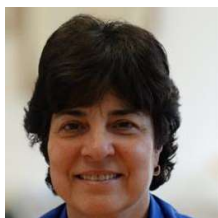
Joni L. Rutter, Ph.D., is the director of the National Center for Advancing Translational Sciences (NCATS) at the National Institutes of Health (NIH). Rutter oversees the planning and execution of the Center's complex, multifaceted programs that aim to overcome scientific and operational barriers impeding the development and delivery of new treatments and other health solutions. Under her direction, NCATS supports innovative tools and strategies to make each step in the translational process more effective and efficient, thus speeding research across a range of diseases, with a particular focus on rare diseases. By advancing the science of translation, NCATS helps turn promising research discoveries into real-world applications that improve people's health. In her previous role as the NCATS deputy director, Rutter collaborated with colleagues from government, academia, industry, and nonprofit patient organizations to establish robust interactions with NCATS programs.

### **Filippa Lentzos**



Dr Filippa Lentzos is Reader (Associate Professor) in Science & International Security at King's College London, where she is jointly appointed in the Department of War Studies and the Department of Global Health & Social Medicine. A biologist and social scientist by training, Dr Lentzos's research critically examines biological threats, health security, biorisk management and biological arms control, and she has written widely on these issues. Dr Lentzos serves as the Director of the King's MA in Science & International Security. She is also an Associate Senior Researcher at the Stockholm International Peace Research Institute (SIPRI), a Non-Resident Scholar at the James Martin Center for Nonproliferation Studies (CNS), and she serves as the NGO Coordinator for the Biological Weapons Convention.

### **Ephrat Levy-Lahad**



Ephrat Levy-Lahad is Professor of Medical Genetics and Internal Medicine at the Hebrew University of Jerusalem and Director of the Genetics Institute at Shaare Zedek Medical Center (SZMC) in Jerusalem, Israel. Professor Levy-Lahad's clinical laboratory includes a large pre-implantation diagnosis service and cancer genetics diagnostics. Her research focuses on population genetics of breast/ovarian cancer and on the genetic basis of rare diseases. Her work on population genetics of breast cancer has led to the recent adoption of population screening for BRCA among Ashkenazi Jews in Israel. Professor Levy-Lahad is also active in bioethical aspects of genetic research. She was co-Chair of Israel's National Bioethics Council and was a member of the USA National Academy of Sciences Committee on gene editing. In 2018 she was awarded Israel's EMET Prize in Life Sciences. She currently serves as Vice President of the Israel National Academy for Science in Medicine.



### **Katherine Littler**



Katherine Littler is currently the co-lead of the Health Ethics & Governance Unit at WHO Headquarters, Geneva. She is passionate about embedding ethics more effectively in global health decision making and increasing ethics capacity around the globe. Whilst a lot of her current focus is on COVID-related work, she is also focused on realizing the potential benefits of emerging technologies in different settings, with particular reference to human genome editing; the ethics and governance of clinical trial design and identifying gaps in ethical oversight in global health.

### **David Liu**



David R. Liu is the Richard Merkin Professor and director of the Merkin Institute of Transformative Technologies in Healthcare, vice-chair of the faculty at the Broad Institute of MIT and Harvard, the Thomas Dudley Cabot Professor of the Natural Sciences at Harvard University, and a Howard Hughes Medical Institute (HHMI) investigator. Liu's research integrates chemistry and evolution to illuminate biology and enable next-generation therapeutics. His major research interests include the engineering, evolution, and in vivo delivery of genome editing proteins such as base editors and prime editors to study and treat

genetic diseases; the evolution of proteins with novel therapeutic potential using phage-assisted continuous evolution (PACE); and the discovery of bioactive synthetic small molecules and synthetic polymers using DNA-templated organic synthesis and DNA-encoded libraries. Base editing, prime editing, PACE, and DNA-templated synthesis are four examples of technologies pioneered in his laboratory. Liu has been elected to the U.S. National Academy of Sciences, the U.S. National Academy of Medicine, and the American Association for the Advancement of Science. He is the 2022 King Faisal Prize Laureate in Medicine.

### **Angelo Lombardo**



Angelo obtained his PhD in Cellular and Molecular Biology in 2011 working on the development of innovative gene therapy approaches based on artificial nucleases and targeted genome editing. His early studies were the first to report gene editing in therapeutically relevant cell types, including human pluripotent stem cells, primary T cells, epidermal stem cells, and neural stem cells. Currently, Angelo is Professor of tissue biology and regenerative medicine at the Vita-Salute San Raffaele University (UniSR; Milan, IT) and Group Leader at the San Raffaele-Telethon Institute for Gene Therapy (SR-Tiget; Milan, IT). He is

also co-founder of Chroma Medicine, a start-up company that aims at developing novel gene therapy modalities based on targeted epigenetic editing. He received several international prestigious awards, including the Young Investigator Award from the European Society of Gene and Cell Therapy and the Excellence in Research Award from the European Society of Gene and Cell Therapy.

### **Tippi MacKenzie**



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 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. Tippi has been elected to the American Society for Clinical Investigation and the National Academy of Medicine for her innovative work.

### **Mike McCune**



Joseph ("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. In recent years, he has helped to form multidisciplinary, collaborative research teams to find a cure for HIV disease, first in the context of NIH- and amfAR-funded "collaboratories" at UCSF (2010-2016) and then as Head of the HIV Frontiers Program at the Bill & Melinda Gates Foundation (2018-present). Throughout this time, he has taken care of

patients with HIV disease at the San Francisco General Hospital AIDS Clinic/Ward 86 and has also actively mentored graduate students and postdoctoral fellows, many of whom have gone on to successful careers in academia or biotech/pharma. He is a member of many scientific and professional societies, including the American Society for Clinical Investigation, the American Association of Physicians, and the Henry Kunkel Society.

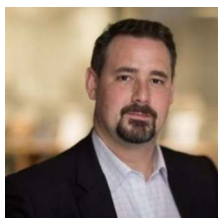
### **Annarita Miccio**



Annarita is a Lab Director of chromatin and gene regulation during development. Dr. Miccio's main interests are the transcriptional control of hematopoiesis, and the development of therapeutic approaches to  $\beta$ -hemoglobinopathies. As a PhD student, she generated a lentiviral vector (LV) successfully used in an early clinical trial for  $\beta$ -thalassemia. As a post-doc and later as an assistant professor, she gained experience in the gene regulation during erythroid development and in evaluating the efficacy of gene therapy approaches for hematopoietic disorders. In 2014, she was appointed as a Lab Director at the Imagine Institute,

where she pursued her studies on transcriptional regulation in normal and diseased stem cells, and their progeny. These basic research studies were instrumental in developing novel strategies for  $\beta$ -hemoglobinopathies. In particular, she optimized the design of a LV currently employed in a clinical trial for sickle cell disease and developed CRISPR/Cas9 strategies for  $\beta$ -hemoglobinopathies. Dr. Miccio is the author of 50 publications and 13 patents. She has coordinated/participated in numerous projects, e.g., funded by ANR, AFM and EU (ERC, HORIZON-RIA and HORIZON-Pathfinder). For her studies, she has received the Sanofi Innovation and the ASGCT Outstanding New Investigator Awards.

### **Piers Millett**



Piers D. Millett, Ph.D. is Executive Director of the International Biosecurity and Biosafety Initiative for Science (IBBIS). IBBIS is a new international organization dedicated to strengthening global biosecurity norms and developing innovative tools and incentives to uphold them. Dr. Millett is a certified biorisk management professional, with a specialization in biosecurity. Dr. Millett was Deputy Head of the Implementation Support Unit for the Biological Weapons Convention (BWC), a treaty for which he worked for over a decade. He has consulted for the World Health Organization, supporting their integration of research and development

into responses to public health emergencies and considering the health implications of advances in technology. As Vice President for Responsibility for iGEM Foundation (International Genetically Engineered Machines Competition), Dr. Millett established and ran a program strengthening the culture of responsibility and risk management with more than 300 projects led by more than 4000 young scientists and engineers from 45 countries across every inhabited continent.

### **Shoukhrat Mitalipov**



Dr. Shoukhrat Mitalipov earned his Ph.D. in Human Genetics in 1994 from the Research Center for Medical Genetics in Moscow. Currently, Dr. Mitalipov is a director of Center for Embryonic and Gene Therapy at Oregon Health & Science University. His research is focused on investigating and developing novel cell and gene therapy approaches for treatment of female infertility.

Another research topic in his laboratory is investigating novel germ line gene therapy approaches that would allow repairing gene defects in gametes or early preimplantation embryos and thus prevent passage of thousands of heritable genetic disorders from parents to their children. Dr. Mitalipov's laboratory is exploring applications of gene editing and gene replacement strategies in preclinical and clinical studies demonstrating feasibility, efficacy and long-term safety of these approaches.

### **Lluís Montoliu**



Lluís Montoliu holds a PhD in Biology and he is a senior researcher and deputy director at the Spanish National Biotechnology Center (CNB-CSIC), a member of the Steering Committee of the Spanish Center for Network Biomedical Research in Rare Diseases (CIBERER) and Honorary Professor at the Madrid Complutense University (UCM). In addition to being a first-line researcher in albinism, he accomplishes numerous activities of scientific outreach and dissemination, and he is a passionate supporter of patient involvement in research.

Lluís Montoliu is a pioneer in the introduction, use and dissemination of CRISPR technology in Spain. His research focuses on the use of CRISPR-genetically edited animals to improve the existing knowledge about albinism.

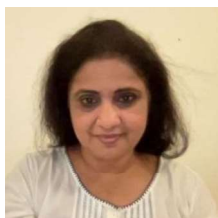
### **Kiran Musunuru**



An actively practicing cardiologist and committed teacher, Kiran Musunuru, M.D., Ph.D., M.P.H., M.L., is Professor of Cardiovascular Medicine and Genetics in the Perelman School of Medicine at the University of Pennsylvania. His research focuses on the genetics of heart disease and seeks to identify genetic factors that protect against disease and use them to develop new therapies. He is a recipient of the Presidential Early Career Award for Scientists and Engineers from the White House, the American Heart Association's Award of Meritorious Achievement and Joseph A. Vita Award, the American Philosophical Society's

Judson Daland Prize for Outstanding Achievement in Clinical Investigation, the American Federation for Medical Research's Outstanding Investigator Award, and Harvard University's Fannie Cox Prize for Excellence in Science Teaching. He recently served as Editor-in-Chief of the scientific journal *Circulation: Genomic and Precision Medicine*. He is author of *The CRISPR Generation: The Story of the World's First Gene-Edited Babies* and *Genome Editing: A Practical Guide to Research and Clinical Applications*. He is co-founder and Senior Scientific Advisor of Verve Therapeutics.

### **Sarojini Nadimpally**



Sarojini Nadimpally is a social scientist and public health practitioner who has been working on Gender, health, bioethics and human rights for over 25 years and is also one of the founder members of Sama Resource Group for Women and Health. She has an academic background in Social sciences, Food & nutrition and public health.

As part of the People's Health Movement (PHM) – an international coalition of academic and civil society health activists working to promote 'Health for All – and founder of Sama Resource Group for Women & Health, she has been working on various issues surrounding public health including, strengthening health systems, health policy, sexual and reproductive health, reproductive and biotechnologies, clinical trials and access to medicines, conflict and health through research, policy advocacy and building solidarity between health and other social movements.

### **Kathy Niakan**



Kathy Niakan is Mary Marshall and Arthur Walton Professor of the Physiology of Reproduction and Director of the Centre for Trophoblast Research at the University of Cambridge from 2020. She is Chair of Cambridge Reproduction, an interdisciplinary strategic research initiative bringing together expertise from 42 different departments, faculties and associated institutions to offer fresh perspectives on reproduction and to engage with the public on sensitive areas of research. From 2021, she has been an Honorary Group Leader at the Babraham Institute and Affiliate Member of the Cambridge Stem Cell Institute.

She is a Group Leader at the Francis Crick Institute, formerly the National Institute for Medical Research, since 2013. Prior to this she was a Centre for Trophoblast Research Next Generation Research Fellow in Cambridge. She obtained a B.Sc. in Cell and Molecular Biology and a B.A. in English Literature from University of Washington. She obtained her PhD at University of California, Los Angeles. She undertook postdoctoral training at Harvard University.

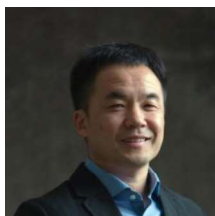
### **Simon Niemeyer**



Simon Niemeyer is Professor and co-founder of the Centre for Deliberative Democracy and Global Governance, and project leader of the Global Citizens' Assembly on Genome Editing. His research focusses on deliberative democracy, using empirical findings to inform its theoretical foundations and how they inform practical democratic innovations. His recent involves conceptualising, measuring, and analysing deliberative reasoning, with implications for the understanding the ethics of epistemology, the nature of deliberative capacity, the role of expertise, and distributed reasoning in

deliberative systems. Much of his work is applied to environmental issues, including climate change, but also covers a broad range of topics including technological development, medical ethics, energy futures, immigration, and parliamentary reform.

### **Yuyu Niu**



Yuyu Niu, Ph.D., is a professor in life sciences, and is now the Vice-chancellor of Kunming University of Science and Technology, China. He is also the Dean of Faculty of Life Science and Technology, Kunming University of Science and Technology, China and the Associate Director of State Key Laboratory of Primate Biomedical Research, China. Research in Yuyu Niu's laboratory is focused on primate pre- and post- implantation embryo development, which play a significant role in cell differentiation and organ formation and therefore has an enormous impact on human diseases. Built on the strength in non-human

primate research, the Yuyu Niu's laboratory has expanded its capability to use a variety of state-of-the-art techniques, including CRISPR/Cas9-based genomic editing, somatic cell nuclear transfer, single cell transcriptomics, cell lineage tracing and stem cells to understand the molecular events at the beginning of primate life and cell reprogramming.

### **Eric Olson**



Eric Olson is the founding Chair of the Department of Molecular Biology at UT Southwestern Medical Center. He also directs the Hamon Center for Regenerative Science and Medicine and the Wellstone Center for Muscular Dystrophy Research at UT Southwestern. He holds the Robert A. Welch Distinguished Chair and the Annie and Willie Nelson Professorship in Stem Cell Research. Eric Olson and his trainees discovered many of the key genes and mechanisms responsible for development and disease of the heart and other muscles. His most recent work has provided new strategies for correction of

genetic disorders of muscle and the heart using CRISPR gene editing. Dr. Olson is a member of the U.S. National Academy of Sciences, the Institute of Medicine, and the American Academy of Arts and Sciences and has received numerous additional honors for his work. He has co-founded multiple biotechnology companies to design new therapies for heart and muscle disease.



### **Kelly Ormond**



Kelly Ormond is a genetic counselor (US ABGC certified) and ELSI researcher. She received her MS in Genetic Counseling from Northwestern University (1994) and a post-graduate certificate in Clinical Medical Ethics from the MacLean Center at the University of Chicago (2001). She joined the Health Ethics and Policy Lab as a Senior Scientist in February 2021, and is an Adjunct Professor in the Department of Genetics at Stanford School of Medicine, Stanford University, California, USA .

Kelly currently serves on the ESHG Program Committee. She is also the current President of the Transnational Alliance for Genetic Counseling (TAGC). Kelly's recent research focuses on patient decision making, the role of uncertainty in decision making, consent and disclosure of genetic test results, personalized medicine, and the interface between genetics and disability.

### **Kyle Orwig**



Dr. Orwig is a Professor of Obstetrics, Gynecology and Reproductive Sciences at the University of Pittsburgh School of Medicine. He is also the director of the Fertility Preservation Program and the Center for Reproduction and Transplantation at Magee-Womens Hospital of UPMC. Research in the Orwig laboratory focuses on stem cells, germ lineage development, fertility and infertility. Our progress investigating reproductive function in fertile individuals provides a basis for understanding the mechanisms of infertility caused by disease, medical treatments, genetic defects or aging. Infertility impacts one in

seven couples in the United States and can have a devastating impact on relationships, emotional well-being and overall health. The Orwig lab is ideally located in Magee-Womens Research Institute and Magee-Womens Hospital of the University of Pittsburgh and is committed to translating lab bench discoveries to the clinic for diagnosis, prevention and treatment of infertility.

### **César Palacios-González**



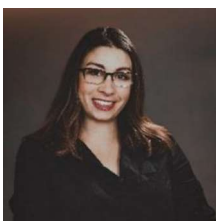
Dr César Palacios-González is a Senior Research Fellow in Practical Ethics at the Oxford Uehiro Centre for Practical Ethics, a Research Fellow at Wolfson College, a Knowledge Exchange Fellow at The Oxford Research Centre in the Humanities, a Senior Associate at Pembroke College, and the Deputy Director of the MSt in Practical Ethics. His research interests include philosophy of medicine, applied ethics, and reproductive ethics.

### **Linda Partridge**



Linda Partridge works on the biology of ageing. Her research is directed to understanding the mechanisms by which health during ageing can be increased in laboratory model organisms and humans. Her work has focussed in particular on the role of nutrient-sensing pathways and diet, and her primary interest is in geroprotective drugs. She is the recipient of numerous awards, was honoured with a DBE for Services to Science in 2009 and is a Fellow of the Royal Society. She is the founding director of the Max Planck Institute for Biology of Ageing and the Biological Secretary of the Royal Society.

### **Alex Pearlman**



Alex Pearlman is a bioethicist and science communicator whose work focuses on the intersection of justice, health policy, and emerging biotechnologies, specifically within the nascent DIYbio/Community Bio movements. She was formerly a researcher in the Wexler Lab at the University of Pennsylvania's Dept. of Medical Ethics and Health Policy, and is a Research Affiliate with the MIT Media Lab's Community Biotechnology Initiative. Pearlman's reporting and analysis has been featured widely in the international press in outlets such as *Stat News*, *New Scientist*, *the MIT Technology Review*, and others. She is currently the Communications Director at Concentric by Ginkgo, the public health and biosecurity arm of synthetic biology company Ginkgo Bioworks.

### **Steve Pearson**



Steven D. Pearson, MD, MSc is the Founder and President of the Institute for Clinical and Economic Review (ICER). Dr. Pearson is also a Lecturer in the Department of Population Medicine at Harvard Medical School. An internist, health services researcher, and ethicist, Dr. Pearson has served in many advisory and leadership roles in academia and government. In 2004 he was awarded an Atlantic Fellowship from the British Government and chose to serve as Senior Fellow at the National Institute for Health and Clinical Excellence (NICE). Returning to the United States in 2005 he was asked to serve during the George W. Bush Administration as Special Advisor, Technology and Coverage Policy, at the Centers for Medicare and Medicaid Services.

### **Yaojin Peng**



Dr. Yaojin Peng, Zhiyi Professor, Institute of Zoology (Chinese Academy of Sciences, CAS), Beijing Institute of Stem Cell and Regenerative Medicine (BISCRM); Director of the Centre for Ethics of Science and Technology (CEST); Director of the Life Sciences and Medical Ethics Committee of the CAS. He holds a bachelor's degree in life science and a Master's degree in Law. Dr. Peng's research focuses on biotechnology law and bioethics, intellectual property rights (patents) and standards, science and technology policy and management.

He currently is a member of the expert committee of China Stem Cell and Regenerative Medicine Collaborative Innovation Platform; Deputy Secretary General of the Academic committee of the Center for Science & Technology Development and Governance, Tsinghua University; a member of the Standard Working Group of Chinese Society for Stem Cell Research (CSSCR)

### **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 (most notably sickle cell disease) but also of other organ systems as well. 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 has been a scientific founder of CRISPR Tx and an academic founder of Graphite Bio and serves on several SAB's. He is a strong advocate for assuring that the next generation of transformative medicines reaches the global community in partnership with those communities.

### **Kaushik Rajan**



capitalism.

Kaushik is a Professor of Anthropology and of Social Sciences and Co-Director of the Chicago Center for Contemporary Theory. His work lies at the intersection of Medical Anthropology and Science and Technology Studies (STS), with commitments to social theories of capitalism and postcolonial studies. Throughout his career, he has had a three-fold set of intellectual commitments: (a) to explore the nature of scientific knowledge, practice, and institutionalization; (b) to elucidate political economic structures that operate across multiple scales using ethnography; and (c) to theorize contemporary

### **Tina Rulli**



Tina Rulli is an Associate Professor in the department of Philosophy at the University of California, Davis. She works in both ethical theory and bioethics, with a focus on reproductive and population ethics. Her published work includes pieces in the *Journal of the American Medical Association*, *Bioethics*, the *Kennedy Institute of Ethics Journal*, and the *Hastings Center Report*. She received her postdoctoral training in bioethics at the National Institutes of Health Clinical Center Bioethics Department and her PhD in Philosophy from Yale.

### **Bettina Ryll**



sustainable innovation.

Dr. Ryll holds a medical degree from the Free University of Berlin, Germany and a PhD in Biomedical Sciences from University College London, UK. After losing her husband to melanoma, she founded the Melanoma Patient Network Europe and developed a special interest in patient-centric clinical research, in particular, innovative trial designs and novel drug development concepts, such as MAPPS (medicines' adaptive pathways to patients), previously known as Adaptive Licensing. Lately, her focus has moved to sustainable healthcare models ensuring access to innovative therapies for cancer patients and incentives for

Dr. Ryll is involved in numerous initiatives promoting evidence-based advocacy. She is fascinated by the enormous potential and capacity of patient networks to both educate and support patients as well as to capture data at the primary data source – the patients themselves – and to generate evidence at a granular level non-accessible to outsiders

### **Mitinori Saitou**



Saitou has been promoting studies on the developmental mechanisms of germ cells, the origin of all life. He clarified the formation mechanisms of mouse germ cells and successfully generated primordial germ cell-like cells (PGCLCs) in vitro from mouse ESCs and iPSCs to produce sperm, oocytes and healthy offspring. He used this experimental system as a model to investigate the molecular mechanisms of key phenomena in germ cell development, including epigenomic reprogramming, oocyte differentiation, and meiosis induction mechanisms. Saitou's analysis of the developmental mechanisms of cynomolgus monkeys has allowed us to identify the characteristics of pluripotent cell lineages in mice, monkeys and humans and the formation mechanisms of germ cells in primates. He has also generated PGCLCs and oogonium from human iPSCs and pioneered research on in vitro reconstitution in the process of human germ cell development.

### **Arafa Salim Said**



Arafa is a sickle cell warrior and has been working passionately in sickle cell disease advocacy in Tanzania and internationally for over 12 years. She is the founder of Sickle Cell Disease Patients Community of Tanzania, which was the first sickle cell disease patient advocacy organization in the country. This group is primarily focused on spreading sickle cell disease awareness, educating families affected by sickle cell disease, and provided hope and social support. Through digital outreach and in-person visits to sickle cell disease clinics, Arafa uses her own life experiences as a sickle cell warrior to offer advice and encouragement. She has served as a patient representative for medical conferences as well as community advocacy events across many countries. Arafa has been recognized for her work as a community advocate and educator, including as 2017 recipient of the International Sickle Cell Advocate of the year Award by Sickle Cell 101 in the United States. In recent years, she has turned her attention to building partnerships with other organizations fighting sickle cell around the world, especially in Africa. In early 2020, she participated in the launch of the East African Sickle Cell Alliance as the Tanzanian representative, alongside advocates and warriors from Kenya and Uganda. She is enthusiastic about participating in the growth of the Umoja Sickle Cell Foundation (UMASCCO) in her role as secretary.

### **Natacha Salomé Lima**



Natacha Salomé Lima is a psychologist who lives in the province of Buenos Aires, Argentina. During 2015-2016 she finished the Master of Bioethics from the Erasmus Mundus Program (KU Leuven, Università degli Studi di Padova, and Radboud Universiteit Nijmegen). She holds a Ph.D. in psychology from the University of Buenos Aires (UBA) with a thesis that explores bioethical dilemmas through a narrative paradigm. Since 2019 Natacha coordinates the postgraduate studies of Psychology in Human Assisted Reproduction at the Argentine Society of Reproductive Medicine (SAMeR). Her research interest includes narrative bioethics, assisted reproductive technologies, and ethical challenges posed by biotechnological developments. She is conducting her research at the National Scientific and Technical Research Council – CONICET Argentina.

### **Sonja Schrepfer**



Dr. Schrepfer is Professor at the University of California San Francisco (UCSF), Gladstone-UCSF Institute of Genomic Immunology, and a Scientific Founder and SVP (Head of the Hypoimmune Platform) of Sana Biotechnology, Inc. Work by Dr. Sonja Schrepfer is at the forefront of stem immunobiology and paves the way for treatment of a wide range of diseases – from supporting functional recovery of failing myocardium to the derivation of other cell types to treat diabetes, blindness, cancer, lung, neurodegenerative, and related diseases. Her work demonstrates that protecting transplanted cells from immune rejection is the key to unlocking the potential of regenerative medicine.

### **Sofonias Tessema**



Sofonias Tessema is the Program Lead for Pathogen Genomics at the Africa CDC. Sofonias received a PhD degree from the Walter and Eliza Hall Institute at the University of Melbourne and was a postdoctoral scholar at the University of California in San Francisco. Sofonias is passionate about capacity building for the implementation of genomic and digital epidemiology for public health in Africa.



### **Alexis Thompson**



Dr. Alexis Thompson is Chief, Division of Hematology at the Children's Hospital of Philadelphia. She is also Professor of Pediatrics at the University of Pennsylvania Perelman School of Medicine and holds the Elias Schwartz MD Endowed Chair in Hematology. Her research interests include hemoglobinopathies (thalassemia and sickle cell disease), and stem cell transplantation in pediatric patients, including gene therapy. Her most significant scientific contributions are clinical and translation studies to better understand and treat hemoglobinopathies, leading to landmark findings in the field. As a leader of the American Society of Hematology, Dr. Thompson helped to develop a comprehensive report on the current state of clinical care for SCD in an effort to identify unmet medical needs, launch a national sickle cell data collection platform and create a sickle cell learning community to improve outcomes. She is also leading efforts to implement newborn screening and early intervention efforts in sub-Saharan Africa, where the burden of SCD is profound.

### **John Tisdale**



John Tisdale received his medical degree from the Medical University of South Carolina in Charleston after obtaining his B.A. in Chemistry from the College of Charleston. He joined the Molecular and Clinical Hematology Branch of NHLBI in 1998 and is now the Chief of the Cellular and Molecular Therapeutics Branch. In 2020 the College of Charleston recognized Dr. Tisdale as one of their Top 25 History makers in honor of the schools 250-year anniversary and was Samuel J. Heyman Service to America Medal finalist. He was recently elected to the American Society for Clinical Investigation and is a member of the American Society of Hematology. He serves as an editorial board member of the journals Stem Cells, Experimental Hematology, and Molecular Therapy Methods & Clinical Development. He has served on the NIDDK/NIAMS Institutional Review Board for over 15 years, is a founding member of the NIH Bone Marrow Transplant Consortium, and is an active member of the NIH Intramural Gene Therapy Task Force. Dr. Tisdale's research and clinical work center on sickle cell disease. His group focuses on developing curative strategies for sickle cell disease through transplantation of allogeneic or genetically modified autologous bone marrow stem cells. He has published over 200 first- and co-author publications.

### **Leigh Turner**



Leigh Turner is the Executive Director of the University of California, Irvine's Bioethics Program and a Professor in the Program in Public Health's Department of Health, Society, and Behavior. He is also a member of UCI's Stem Cell Research Center and serves as Ethics Lead for UCI's Institute for Clinical and Translational Science. Turner's current research addresses ethical, legal, and social issues related to stem cells and regenerative medicine products. In particular, he uses approaches from health ethics and the social sciences to study the activities of clinics engaged in direct-to-consumer marketing of unproven and unlicensed cell-based interventions. He has also written about ethical dimensions of health-related travel, crowdfunding for medical interventions, and end-of-life care in multicultural societies.

### **Fyodor Urnov**



Fyodor Urnov is a Professor of Molecular Therapeutics at UC Berkeley and a Scientific Director at its Innovative Genomics Institute. He co-developed the toolbox of human genome and epigenome editing and led the team that developed a strategy for genome editing in the hemoglobinopathies, sickle cell disease and beta-thalassemia, that has yielded sustained clinical benefit for subjects in several ongoing clinical trials. At the IGI Fyodor directs efforts to develop scalable CRISPR-based approaches to treat diseases of the immune system, sickle cell disease, neurodegeneration, and neuroinflammation. His recent op-ed in the New York Times describes a major goal for the field of genomic therapies and a key focus of Fyodor's work at the IGI - expanding access to CRISPR-based treatments for N=1 genetic disease.

### **Amy Wagers**



Amy Wagers is the Forst Family Professor of Stem Cell and Regenerative Biology, as well as the co-chair of the Department of Stem Cell & Regenerative Biology, at Harvard University, Senior Investigator in the Section on Islet Cell and Regenerative Biology at the Joslin Diabetes Center, an HHMI Early Career Scientist, a 2018 NIH Directors' Pioneer Award recipient, and a member of the Paul F. Glenn Laboratories for the Biological Mechanisms of Aging at Harvard Medical School. Dr. Wagers received her PhD in Immunology and Microbial Pathogenesis from Northwestern University and completed postdoctoral training in stem cell biology at Stanford University.

### **Ambroise Wonkam**



Ambroise is Professor of Genetic medicine and Director of the Mckusick-Nathan Institute and Department of Genetic Medicine at the Johns Hopkins University School of Medicine, Baltimore, USA. He is also Professor of Genetic Medicine and Director of GeneMAP (Genetic Medicine of African Populations) Research Center, University of Cape Town, South Africa. Born in Yaoundé, Cameroon, Ambroise Wonkam grew up witnessing the pain, disability, and death caused by sickle cell disease – among family friends, classmates, and colleagues. Very early in his medical education that experience led him to cell biology and, later, to genetics. Focusing on sickle cell disease seemed, then, almost like a calling. Wonkam has won multiple prizes, including the prestigious 2020 Alan Pifer Award. In 2014, he won the competitive Clinical Genetics Society International Award from the British Society of Genetic Medicine. He is the president of the African Society of Human Genetics.

### **Dagan Wells**



Dagan Wells has been involved in preimplantation genetic testing (PGT) and the study of gametes and early embryos for three decades. Throughout his career he has been at the forefront of new developments in the field, pioneering the application of advanced methodologies, including genome editing, meiotic spindle and pronuclear transfer, whole genome amplification and next generation sequencing. Dagan is currently professor of reproductive genetics at the University of Oxford and also directs Juno Genetics, a state-of-the-art genetics laboratory specializing in PGT, non-invasive prenatal testing, carrier screening and other genetic tests related to infertility treatment and pregnancy. His work has led to the publication of over 200 scientific papers and has been recognized with multiple awards. He has been acknowledged with fellowships of the Royal College of Pathologists, the Royal Society of Biology, the Royal College of Obstetricians and Gynaecologists and the Institute of Biomedical Science. Dagan is the incoming President of the Preimplantation Genetic Diagnosis International Society.

### Ping Yan



Dr. Ping Yan is assistant professor at Dalian University of Technology, visiting scholar at Delft University of Technology (2012; 2014). Her research focuses on the ethics of science and technology, responsible research and innovation (RRI), especially focus on the ethics of genome editing technology in recent years. Recently she has won a National Social Science Fund with the project title of “Ethical Governance of Human Genome Editing in China from the Perspective of Global Governance” (2022-2025).

She has organized a research team working on the ethics of Human Genome Editing in China with four outstanding young scholars, and is participating the Third International Summit as a team, including Dr. Lu Gao, associate professor of STS in the Institute for the History of Natural Sciences, Chinese Academy of Sciences; Dr. Yang Yang, executive editor-in-chief of *Journal of Medicine and Philosophy* in China and associate professor of medical ethics in Dalian Medical University; Dr. Wenlong Lu, vice dean and associate professor of philosophy of technology at Dongbei University of Finance and Economics; Dr. Wei Zhang, professor of philosophy of technology at Dalian University of Technology.

### Joy Zhang



Joy Y. Zhang is Reader in Sociology and the Founding Director of the Centre for Global Science and Epistemic Justice at the University of Kent. Originally trained as a medical doctor, her research investigates the transnational governance of scientific uncertainty and the democratisation of knowledge production. Conceptually, her work contributes to sociological theories of risk, cosmopolitanism, decolonisation and subaltern politics. She has undertaken empirical studies on stem cells, synthetic biology, genome editing, food movements and environmental politics. She is the author of four academic

monographs: *The Cosmopolitanization of Science: Stem Cell Governance in China*(2012), *Green Politics in China: Environmental Governance and State-Society Relations*(2013), *The Elephant and the Dragon in Contemporary Life Sciences: A Call for Decolonising Global Governance* (2022) and *Democratic Participation and the Cosmopolitics of Science: Why Scientific Citizenship Matters in the 21st Century* (forthcoming).