Nominations for Appointment to the Grants Working Group (GWG)

NEW APPOINTMENTS

Theresa Alenghat, VMD, PhD
Associate Professor, Cincinnati Children's Hospital Medical Center

Referral: Dr. Alenghat was identified by Drs. Linda Nevin and Hayley Lam.

Expertise Relevance to CIRM GWG: Dr. Alenghat's expertise in inflammation, immunobiology, and tolerance will be invaluable in reviewing Discovery program applications.

Prior Service in CIRM Reviews: Dr. Alenghat has participated in Discovery stage program reviews.

Bio:
Dr. Theresa Alenghat is an Associate Professor in the Immunobiology Division of Cincinnati Children's Hospital Medical Center at the University of Cincinnati. Her research program explores mechanisms underlying the host-microbiota relationship, and how this regulation affects intestinal immunity, repair, and inflammation. Dr. Alenghat has pioneered studies revealing that epigenetics and histone deacetylases permit intestinal epithelial cells to sense commensal bacteria and convey this information to the mammalian host. Her lab's work includes investigation into pathways that enable diet and microbiota-derived metabolites to direct epithelial and immune cell responses during health and disease.

Dr. Alenghat earned her VMD and PhD from University of Pennsylvania. She conducted graduate research in a lab focused on diabetes and metabolic disease and completed her residency in Anatomic Pathology. She has authored over 40 articles in journals such as *Nature*, *Science*, *Cell Host & Microbe*, *Journal of Clinical Investigation,* and *Immunity*. Dr. Alenghat is the recipient of awards from the NIH, Burroughs Wellcome Fund, Kenneth Rainin Foundation, Pew Charitable Trust, Crohn's & Colitis Foundation, and the American Gastroenterological Association.

Christine Kay, MD
Vitreoretinal Surgeon and Director of Electrophysiology and Retinal Genetics, Vitreoretinal Associates

Referral: Dr. Kay was identified by Drs. Lila Collins and Hayley Lam.

Expertise Relevance to CIRM GWG: Dr. Kay's expertise with vitreoretinal surgery, subretinal gene therapy trials, and inherited retinal disease will be invaluable in reviewing Discovery, Translational, and Clinical program applications.

Prior Service in CIRM Reviews: Dr. Kay has participated in Discovery stage program reviews.

Bio:
Dr. Christine Kay, a vitreoretinal surgeon, joined Vitreoretinal Associates in Gainesville, Florida in the fall of 2014. She is the director of electrophysiology and retinal genetics Vitreoretinal Associates, involved as principal investigator (PI) in 15 IRD clinical trials currently. Prior to this, she was an Assistant Professor and Director of the retinal fellowship and retinal genetics service at the University of Florida. She is actively involved in multiple subretinal gene therapy trials and interested in optimizing surgical techniques for subretinal delivery. She has a large IRD patient population, with over 900 patients in her clinical database and with 700 of these patients genotyped.

Dr. Kay graduated magna cum laude from Harvard University with a degree in neuroscience and went to medical school at the University of Florida. She completed her ophthalmology residency at the University of South Florida where she was chief resident, and completed her vitreoretinal fellowship training at the University of Iowa. During her fellowship at University of Iowa, she developed a particular interest in inherited retinal disease (IRD). After completion of her fellowship, she was awarded a 5-year Career Development Award from the Foundation Fighting Blindness (FFB), which funded research (both basic science and clinical) focusing on the development and optimization of a viral vector delivery system for the genetic treatment of achromatopsia. She is a PI and surgeon in the AGTC-sponsored gene therapy treatment trials for both CNGB3 and CNGA3 associated achromatopsia. She was also PI and surgeon for Biogen/Nightstar sponsored XIRIUS gene therapy study for XLRP as well as 4D Therapeutics trial for RPGR associated RP, and is a PI now for Meira-sponsored phase 3 gene therapy study for XLRP. She is also a PI in the Alkeus-sponsored Stargardt disease trial, as well as the Foundation Fighting Blindness sponsored natural history trials on patients with Ush2A-associated Usher syndrome (RUSH2A) and EYS associated RP. She is also a PI for Iveric Bio sponsored trial for Stargardt disease. Dr. Kay is also PI and surgeon for RegenxBio gene therapy trial for exudative AMD, Gyroscope's gene therapy trial for atrophic AMD, and 4D Therapeutic's trial for exudative AMD. She is a member of Macula Society and Retina Society.

Christopher Mayhew, PhD
Director, Pluripotent Stem Cell Facility at Cincinnati Children's Hospital Medical Center

Referral: Dr. Mayhew was identified by Dr. Hayley Lam and Dr. Uta Grieshammer.

Expertise Relevance to CIRM GWG: Dr. Mayhew's expertise with pluripotent stem cells, organoids and core facilities will be invaluable in reviewing Infrastructure and Discovery stage program applications.

Prior Service in CIRM Reviews: N/A

Bio:
Dr. Christopher Mayhew is an Associate Professor in the Division of Developmental Biology at Cincinnati Children's Hospital. He also directs the Pluripotent Stem Cell Facility (PSCF), a shared resource laboratory that facilitates and supports human pluripotent stem cell (hPSC) research. Services offered by the PSCF include the generation of patient-derived human induced pluripotent stem cell (iPSC) lines, CRISPR/Cas-mediated iPSC genome editing, and production of 3-dimensional hPSC-derived gastrointestinal organoids. The scientific focus of Dr. Mayhew's work has been largely in the areas of cancer biology, experimental therapeutics and stem cell biology.

Dr. Mayhew received his PhD from the University of Wolverhampton (UK). He completed his post-doctoral training at the University of Kentucky Department of Biochemistry and the University of Cincinnati Department of Cell and Cancer Biology (USA), with the latter completed through the Environmental Carcinogenesis and Mutagenesis Training Program. For his contributions to Cincinnati Children's Hospital Medical Center, he was awarded the CCHMC Junior Faculty Service Achievement Award. He has published over 50 peer-reviewed articles, served as pilot grant reviewer for the Center for Mendelian Genomics and Therapeutics and the Center for Stem Cell and Organoid Medicine, reviewed manuscripts for journals such as *PLoS One, STAR Protocols,* and *Developmental Dynamics,* and chairs the CCHMC Embryonic Stem Cell Oversight (ESCRO) Committee.

Takanori Takebe, MD, PhD
Associate Professor and Endowed Chair of Organoid Medicine, Cincinnati Children's Hospital Medical Center

Referral: Dr. Takebe was identified by Dr. Liz Noblin.

Expertise Relevance to CIRM GWG: Dr. Takebe's expertise with organoid models, engineering strategies, and hepatology will be invaluable in reviewing Discovery and Translational stage program applications.

Prior Service in CIRM Reviews: Dr. Takebe has participated in Discovery stage program reviews.

Bio:
Dr. Takanori Takebe is the Associate Professor and Endowed Chair of Organoid Medicine, Divisions of Gastroenterology, Hepatology and Nutrition & Developmental Biology, at Cincinnati Children's Hospital Medical Center (CCHMC). He also serves as the Director of Commercial Innovation at the CCHMC Center for Stem Cell and Organoid Medicine, and he is an Adjunct Professor & Director of the Communication Design Center at Yokohama City University and Professor at Osaka University in Japan. His research focuses on finding alternative approaches to traditional organ transplantation, particularly on developing an "in-a-dish" engineering strategy for directing complex

hepatogenesis from human stem cells, and doing so by expanding the understanding of liver organogenesis, translating the knowledge into in-a-dish principles to guide organoid self-organization from human pluripotent stem cells, and advancing complex liver modeling with vascular, mesenchymal, immune and endocrine components. He is currently pursuing highly personalized diagnostics and therapeutics development, by establishing en masse population-scale organoid disease modeling approach.

Dr. Takebe earned his MD and PhD in Regenerative Medicine from Yokohama City University, Japan. His surgical internship at Columbia University's' transplant centers, which brought him into contact with terminally ill patients unlikely to receive life-saving donor organs due to lengthy waiting lists, inspired him to pursue a path towards developing alternative liver transplantation options. His ultimate goal is to save children in need of a new liver through the development of organoids. Among other services, he serves as an editorial advisor of *Cell Stem Cell* and has reviewed for *NEJM, Nature, Science, Cell Stem Cell,* and many others. Among many honors, he has received the Robertson Investigator Award, which supports scientists whose pioneering approaches have the potential to transform the field of stem cell research, from the New York Stem Cell Foundation. He is also an elected member of American Society of Clinical Investigation (ASCI), is on the advisory board of Cell Stem Cell, and is on the Board of Directors for the International Society for Stem Cell Research (ISSCR).

Wenli Yang, PhD
Research Assistant Professor of Medicine and Director of the iPSC Core Facility, University of Pennsylvania

Referral: Dr. Yang was identified by Drs. Ross Okamura, Uta Grieshammer, and Hayley Lam.

Expertise Relevance to CIRM GWG: Dr. Yang's expertise with core facilities and iPSC lines/models will be invaluable in reviewing Infrastructure and Discovery stage program applications.
Prior Service in CIRM Reviews: N/A

Bio:
Dr. Wenli Yang is a Research Assistant Professor of Medicine and the Director of the human iPSC Core Facility at the University of Pennsylvania. She is also a member of the Institute for Regenerative Medicine, the Cardiovascular Institute, the Institute for Diabetes, Obesity and Metabolism, and the Institute for Translational Medicine and Therapeutics at University of Pennsylvania. Her research focuses on developing human PSC-derived models of disease, with a particular interest in cellular models of insulin responsive tissues and using these models to elucidate the function of diabetes-associated genes.

Dr. Yang obtained her PhD in Microbiology and Molecular Genetics from UCLA and performed her postdoctoral training in adipose tissue development and function at the Harvard Medical School. Prior to her tenure at University of Pennsylvania, she worked as a discovery scientist for Syndexa Pharmaceuticals on potential therapies for diabetes. She has 15 years of experience leading the University of Pennsylvania iPSC Core Facility, which services internal and external research communities with stem cell technologies including iPSC derivation, genome editing and differentiation. She has worked with over 50 client laboratories and managed over 20 direct reports. She has extensive expertise in the areas of human iPSC derivation, CRISPR/Cas9-mediated iPSC genome editing, lineage-directed differentiation of iPSCs in both 2D and 3D, and in managing the iPSC Core. Among other commitments, she has served as the President (2021-2023) of the Stem Cell COREdinates, an international consortium of PSC-focused core facilities with the goal of sharing expertise with the larger stem cell community.

Ting Zhou, PhD
Director of SKI Stem Cell Research Facility, Memorial Sloan Kettering Cancer Center

Referral: Dr. Zhou was identified by Dr. Hayley Lam.

Expertise Relevance to CIRM GWG: Dr. Zhou's expertise with core facilities, particularly with pluripotent stem cell gene editing and screens, will be invaluable in reviewing Infrastructure program applications.

Prior Service in CIRM Reviews: N/A

Bio:
Dr. Ting Zhou is the Director of SKI Stem Cell Research Facility and an Assistant Lab Member at Memorial Sloan Kettering Cancer Center (MSKCC). As the Director of the Stem Cell Research Facility at the MSKCC, she is fully focused on establishing a hPSC-based research facility with a mission of sharing stem cell expertise and resources. Dr. Zhou was a pioneer in establishing a protocol for using human urinary cells as a source to generate iPSCs in a

noninvasive manner. Utilizing this protocol, she developed a panel of iPSC models for studying premature aging and cardiovascular diseases. She has also combined the hPSC technology with chemical screens and the latest genome editing technologies such as prime editing, base editing, and CRISPR/Cas9 based knock-out and knock-in in hPSCs for her own research and collaboration projects. Her group continually refines and adapts the latest technologies relevant to hPSC research including genetic manipulation, directed differentiation, trans-differentiation and reprogramming, and offer them to the basic and clinical researchers in the community.

Dr. Zhou received her PhD in Stem Cell Biology from the Guangzhou Institutes of Biomedicine and Health, Chinese Academy of Sciences in Guangzhou, China, and she completed her postdoctoral fellowship in Stem Cell Biology at Weill Cornell Medicine. She serves as Scientific Editor of *STAR Protocols,* as an ad hoc reviewer for journals such as *Nature Methods, Nature Communications, STAR protocols, Journal of Cellular Biochemistry, Cellular Reprogramming, Current Stem Cell Research & Therapy, Development Growth and Differentiation* and has reviewed training grants for NYSTEM/CSCB.

REAPPOINTMENTS

CIRM is seeking the reappointment of the individuals listed in the table below. Their updated biographies follow.

Proposed Reappointments to GWG

Last	First	Term	Years	Expertise
Noble	Mark	3	6	Nervous System Injury, Cancer, Lysosomal Storage Diseases, & Drug Discovery

Mark Noble, PhD
Dr. Mark Noble is Professor in Biomedical Genetics and Neuroscience at University of Rochester. He has been involved in multiple aspects of stem cell research since the early 1980s. Currently, his lab is focused on discovering novel cancer treatments that are more efficacious, cost effective, and have fewer off target effects; on treating diseases in pediatric and adult populations for which lysosomal dysfunction plays a prominent role; and on treating acute and chronic damage to the central and peripheral nervous system, including spinal cord injury and peripheral nerve injury.

Dr. Noble earned his BA in Biology and Philosophy from Franklin & Marshall College, and his PhD in Genetics from Stanford University. He completed his postdoctoral training in the MRC Neuroimmunology Project at University College London and has been a faculty member at the Institute of Neurology at Queen Square, the Ludwig Institute of Cancer Research and University College London, the Huntsman Cancer Institute (University of Utah) and the University of Rochester. He has served on the editorial boards of several professional journals, including *Developmental Neuroscience, Experimental Neurology,* and *Current Stem Cell Research & Therapy* and is co-author of over 160 scientific publications. He is an inventor on over 20 awarded or pending patents, has consulted for multiple pharmaceutical and biotechnology companies, and served on the founding scientific advisory board of Acorda Therapeutics, Inc., which succeeded in obtaining FDA approval for enhancing walking ability in people with multiple sclerosis with 4-aminopyridine, a potassium channel blocker. He is currently also a founder and Acting Chief Scientific Officer of Solaxa, Inc., a biotechnology company focused on novel applications of potassium channel blockers in treating and promoting tissue repair in a variety of injuries and diseases.

Dr. Noble has served on the GWG for almost 12 years. He has reviewed for Discovery and Translational stage programs, as well as Education programs, New Faculty programs, Research Leadership programs and Tools and Technology programs.