CIRM
CALIFORNIA'S STEM CELL AGENCY

Nominations for Appointment to the Grants Working Group (GWG)

Appointment of New Members

Andrew Baker, BSc, PhD, FAHA, FESC, FRSE, FMedSci
Andrew graduated from the University of London in 1990 with a First Class BSc (Joint Honours) in pharmacology and toxicology and then studied for his PhD with the Leukaemia Research Fund at the University of Wales College Of Medicine, graduating in 1994. He then joined the group led by Professor Andrew Newby for his post-doctoral work in Cardiff and developed adenoviral vectors for gene delivery studies in the cardiovascular system. He then transferred to a lectureship at the University of Bristol (Bristol Heart Institute) to continue studies on adenovirus-mediated gene transfer to assess vascular function and gene therapy. In 1999, Dr. Baker joined Professor Anna Dominiczak at the University of Glasgow as a Senior Lecturer in Molecular Medicine, then as Reader and in 2005 as Professor of Molecular Medicine.

Andrew was awarded the Blandsford Prize (1990) in pharmacology and the "Update in Thrombolysis Research" (Berlin, 1998) for his publication entitled "Divergent effects of tissue inhibitor of metalloproteinase-1, -2 or -3 overexpression on rat vascular smooth muscle cell invasion, proliferation and death in vitro: TIMP-3 promotes apoptosis" which was published in the Journal of Clinical Investigation. In 1999, he was awarded the British Cardiac Society Young Investigator Research Prize for his work "Gene therapy for vein grafting: Tissue inhibitor of metalloproteinases-3 (TIMP-3) inhibits neointima formation in vitro and in vivo in part by promoting apoptosis". He was awarded the MakDougall-Brisbane prize from the Royal Society of Edinburgh in 2008 and a fellowship from the Society in 2010. Also in 2010 he was awarded an Outstanding Achievement Award from the European Society of Cardiology and in 2011 received a Royal Society Wolfson Research Merit Award.

From August 2010 to November 2011 he was Acting Director of the Institute for Cardiovascular and Medical Sciences at the University of Glasgow and in 2011 he was awarded a British Heart Foundation Chair of Translational Cardiovascular Medicine. In 2015 he was awarded a Fellowship of the Academy of Medical Sciences and from 1st October, 2015 Andrew relocated his BHF Chair to the Centre for Cardiovascular Science at the Queen's Medical Research Institute, University of Edinburgh, UK.

Daniel E. Bauer, MD PhD
Daniel Bauer is a physician-scientist whose research utilizes genome editing to understand the causes of blood disorders and to develop innovative therapeutic strategies. His clinical work in pediatric hematology focuses on the care of patients with hemoglobin disorders. He received his ScB in Biology from Brown University and MD-PhD from the University of Pennsylvania. He completed clinical training in Pediatrics and Pediatric Hematology/Oncology at Boston Children's Hospital and Dana-Farber Cancer Institute. He is a Principal Investigator and Staff Physician at Dana-Farber/Boston Children's Cancer and Blood Disorders Center, Assistant Professor of Pediatrics at Harvard Medical School, Principal Faculty at the Harvard Stem Cell Institute, and Associate Member of the Broad Institute of MIT and Harvard. His honors have included the American Society of Clinical Investigation Young Physician-Scientist Award (2014), NIH Director's New Innovator Award (2016), and Society for Pediatric Research's Young Investigator Award (2017).

Alexey Bersenev, MD, PhD
Dr. Bersenev received his medical education and certification as a general surgeon in Russia. He holds a PhD in transplantation/ pathology. He gained expertise in immunology, hematology, stem cell biology and published scientific papers during post-doctoral training in the US in Philadelphia at the Thomas Jefferson University and the Children's Hospital of Philadelphia. He was trained and worked as a cell manufacturing specialist at the University of Pennsylvania. He was involved in clinical cell processing for multiple clinical trials in a GMP manufacturing facility. He gained expertise in the manufacture of CAR T-cell products for several pioneering clinical trials and technology transfer to industry

Dr. Bersenev has an expertise in manufacturing of cellular products for clinical trials, including product and process development, cell processing and expansion, gene modifications, operations of academic GMP facility and compliance with regulations. Currently, he holds an administrative position as a Laboratory Director of the Cell Therapy Processing Lab and Advanced Cell Therapy Lab at Yale-New Haven Hospital. In addition to his administrative role, he is an Associate Research Scientist at the Department of Laboratory Medicine at the Yale University. His research interests include optimization of T-cell manufacturing, automation of cell processing, cell separation and sorting, regulatory challenges and analysis of clinical trials and industry trends in cell therapy.

Dennis C. Crawford MD, PhD
Dennis Crawford is the Director of Sports Medicine and Associate Professor in the Department of Orthopedics & Rehabilitation at Oregon Health & Science University in Portland, Oregon. He maintains a full-time clinical practice focused on surgical repair and replacement of cartilage, articular and ligamentous soft tissue injury (e.g. rotator cuff, ACL tears, Osteochondritis Dissecans) around the knee and shoulder.

Dr. Crawford received his medical training at Boston University School of Medicine in Boston, Massachusetts where he received a doctorate (PhD) in Biochemistry and his medical degree (MD). This was followed by an Orthopedic Surgery residency at Brown University in Providence, Rhode Island before 2 years of additional Orthopedic Fellowship training. Initially an Orthopedic Trauma & Fracture fellowship at Brown University Hospitals in 2000, and subsequently an Orthopedic Sports Medicine, Knee and Shoulder fellowship at University of California, San Francisco in 2001.

Dr. Crawford has published over 50 scholarly articles related to best practices surrounding knee and shoulder sports medicine surgery and cartilage repair. He is, or has been, the lead Principal Investigator on multiple national FDA regulated clinical trials related to cartilage surgery and maintains a particular interest in new frontiers for cartilage repair and tissue regeneration. He is a founding member of the MOCA (Metrics of OsteoChondral Allograft) study group, an active member of the International Cartilage Repair Society (ICRS), American Orthopedic Society for Sports Medicine (AOSSM), American Association of Orthopedic Surgeons (AAOS) and the Oregon Athletic Association Medical Aspects of Sports Committee.

Corey Cutler, MD, MPH, FRCP(C)
Corey Cutler is an Associate Professor of Medicine at Harvard Medical School, and a Senior Physician in the Division of Hematologic Oncology, Department of Medical Oncology at the Dana-Farber Cancer Institute and Brigham and Women's Hospital, Boston, MA. He is also an Affiliate faculty Member of the Harvard Stem Cell Institute, Cambridge, MA.

Dr. Cutler graduated from McGill University's Faculty of Medicine, completed a residency in Internal Medicine at the McGill University Health Science Center, and completed fellowship training in hematology, medical oncology, and stem cell transplantation at the Dana-Farber Cancer Institute. Dr. Cutler earned an MPH degree at the Harvard School of Public Health.

Currently, Dr. Cutler is on the Board of Directors of the American Society of Blood and Marrow Transplantation serving as Treasurer, and is the Co-Chair of the Clinical Trials Working Group of the NIH Consensus Conference on Chronic GVHD. He recently completed terms as the Co-Chair of the CIBMTR GVHD Working Committee, and as a member of the Clinical Trials Advisory Committee of the CIBMTR. Dr. Cutler is on the editorial boards for the journals Biology of Blood and Marrow Transplantation, the Journal of Clinical Oncology and the American Journal of Hematology. He has been a contributing author on more than 200 peer-reviewed publications and 25 reviews and book chapters. His research focuses on development of novel methods of acute and chronic graft-vs.-host disease prophylaxis and therapy, umbilical cord blood transplantation, and decision theory in stem cell transplantation.

Rachel Gibson, PhD
Rachel Gibson graduated with a PhD in Medicine at the University of Adelaide in December 2004 having focused on a new aspect of supportive care in cancer. Following the completion of her PhD she was awarded a Cancer Council South Australia Post-Doctoral Research Fellowship (2004-2009) to continue her research into gut toxicity at the Royal Adelaide Hospital. In 2008 Professor Gibson took up an

academic position within the School of Medical Sciences at the University of Adelaide. She was quickly promoted in 2010 to Senior Lecturer and in 2014 to Associate Professor. She took up the role of Dean: Academic at the University of South Australia in 2016. Professor Gibson also holds significant leadership roles within the Multinational Association of Supportive Care in Cancer (MASCC). Currently she is sits on the Executive Committee where she holds the role of Secretary. In addition she is the Annual Meeting Chair.

Professor Gibson is currently a Board Member of the Multinational Association for Supportive Care in Cancer (MASCC) having been elected by her international peers at the beginning of 2014. She is also a member of the MASCC Executive Committee (Secretary) as well as the Scientific Chair of the Annual MASCC Meeting. Her role as Scientific Chair involves extensive external stakeholder relationships which need to be managed in conjunction with the delivery of the meeting. Key relationships are also fostered with clinicians, MASCC staff, commercial pharmaceutical companies, the MASCC Board as well as many service suppliers.

Another of Professor Gibson's recent key projects was as the leader for producing the updated Clinical Practice Guidelines for Gastrointestinal Mucositis. Her role was to lead an international team of clinicians, allied health professionals and scientists to produce evidence-based clinical practice guidelines through data collation, analysis, international presentations and finally Clinical Practice Guidelines dissemination. These current guidelines provide clinicians from around the world with tools to effectively manage cancer patients with gastrointestinal mucositis.

In addition, over the past decade Professor Gibson has developed extensive international collaborations with a variety of large pharmaceutical companies. These collaborations have resulted in her appointment as an advisory board member specifically looking at gastrointestinal toxicity following cancer treatment with multiple companies. Professor Gibson has also successfully negotiated and delivered-on-time contract research projects for commercial pharmaceutical companies. Through this exposure to commissioned contract project delivery, she is very accustomed to key elements of project management such as schedule management, delivery risk management, budget management and communication with stakeholders.

Mary Beth Henderson, MBA, PhD
Mary beth Henderson is VP of Regulatory Affairs and Quality Systems and a Senior Principal Advisor at RCRI, Inc. She has worked for over 30 years in medical device and biotechnology industries. She brings experience in strategic regulatory, quality, R&D, and business development roles to her project work. As a Senior Principal Advisor for RCRI, Mary has worked with clients to develop domestic and international regulatory strategies, draft submissions, and negotiate their approval by FDA and/or other regulatory agencies. Leveraging her years of R&D and product development experience, Mary often works with product development teams or early-stage companies during initial phases of product design to provide regulatory support and guidance. Her chemistry and biotechnology expertise help to efficiently support project involving combination products or medical devices incorporating biological materials. Mary has an MBA from the University of Massachusetts and a PhD in Chemistry.

Sadik Kassim, PhD
Sadik Kassim is currently Chief Scientific Officer at Mustang Bio, which is a clinical stage biotech company focused on the development of novel CAR-T therapies in partnership with the City of Hope National Medical Center and the Fred Hutchinson Cancer Research Center. Previously, Sadik was head of Analytical Development for the Cell and Gene Therapies unit at Novartis Pharmaceuticals in Cambridge, Massachusetts where he contributed to the successful BLA filing of CD19 CAR-T therapy (Kymriah) for pediatric ALL and DLBCL. Prior to that, Sadik was a Research Biologist in the Surgery Branch at the National Cancer Institute (NCI) where he contributed to several first in human clinical trials and participated in the manufacture of CAR, TCR, and TIL based cellular immunotherapies for cancer. At the Surgery Branch, Sadik helped with the initial development work that led to Kite's CD19 CAR-T cell therapy product for lymphoma (Yescarta). A viral immunologist by training, Sadik has over a decade of experience in translational research and the development of cell and gene therapies, including *ex vivo* gene modified cell therapies and *in vivo* AAV based gene therapies.

Ulrik B. Nielsen, PhD
Ulrik Nielsen is an experienced entrepreneur, executive and scientist. Dr. Nielson was most recently a founder of Torque Therapeutics where he served as the President and CEO until 2018. Torque is developing an entirely new class of cell therapy for cancer using its proprietary Deep Priming technology. Previously, he was a founder and CSO of Merrimack (NASDAQ: MACK). At Merrimack, the team put six novel anti-cancer therapeutics into clinical development and got the drug Onivyde approved for pancreatic cancer. At Merrimack, Ulrik also led the spinout of Silver Creek Pharmaceuticals (private) where he continues to serve as a board member. Silver Creek is focused on targeting growth factors to specific tissues and cell types to promote their survival and regeneration. The first targeted growth factor is poised to enter clinical development in 2018. He received his M.S. and Ph.D. degrees from the University of Copenhagen in molecular biology and trained at the University of California, San Francisco.

George A. Truskey, PhD
George Truskey is the R. Eugene and Susie E. Goodson Professor of Biomedical Engineering and the Senior Associate Dean for the Pratt School of Engineering at Duke University. He received his PhD in Chemical Engineering in 1985 and has been a member of the faculty at Duke University since 1987. His current research interests include research interests include the cell response of cells to physical forces, adhesion and function of endothelial cells on biomaterials and biological surfaces, cardiovascular tissue engineering, skeletal muscle tissue engineering and the development of microphysiological systems for drug and toxicity testing. From 2003-2011, he was Chair of the Department of Biomedical Engineering at Duke University. During that time, he directed Duke's Translational Research Partnership with the Coulter Foundation and the successful transition to an endowed program. He is the author of over 125 peer reviewed research publications, a biomedical engineering textbook entitled Transport Phenomena in Biological Systems, six book chapters, 1 patent and 2 patent applications. He is a Fellow of the American Association for the Advancement of Science (AAAS), Biomedical Engineering Society (BMES), the American Institute of Medical and Biological Engineering, and the American Heart Association. He was president of BMES from 2008 to 2010. In 2007, he received the Capers and Marion McDonald Award for Excellence in Mentoring and Advising from the Pratt School of Engineering at Duke.

Mitch Weiss, MD, PhD
Mitch Weiss is a physician-scientist who cares for pediatric patients with non-malignant blood diseases and performs related laboratory research. He received his MD and PhD degrees at the University of Pennsylvania School of Medicine, followed by training in pediatrics and pediatric hematology at Boston Children's Hospital, The Dana Farber Cancer Institute and Harvard University. For 15 years, he was a faculty member at The University of Pennsylvania School of Medicine (UPENN) and Children's Hospital of Philadelphia (CHOP) where he rose to the rank of Professor of Pediatrics with Tenure. He was Director for the Pediatric Physician-Scientist Development Program at CHOP (2007-2011) and Associate Director for the Combined Degree and Physician Scholar Program at UPENN (2001-2014). He has mentored numerous fellows and young faculty, 4 of who received NIH K08 development awards. In 2014, he became Chairman of the Hematology Department at St. Jude Children's Research Hospital, where he oversees the Divisions of Experimental Hematology and Clinical Hematology.

Dr. Weiss' research focuses on understanding the biology of blood cell development, particularly red blood cells. Since moving to St. Jude, his work has placed a greater emphasis on translational studies aimed at developing new treatments for hemoglobinopathies, particularly sickle disease and □-thalassemia. Dr. Weiss has published over 125 original research papers in journals that include Blood, Journal of Clinical Investigation, Nature, Nature Genetics, Nature Medicine and Proceedings of the National Academy of Science. He has been funded continually by the National Institutes of Health (NIH) since 2002 and is a permanent member of the NIH study section, Molecular and Cellular Hematology. He is a member of The American Society for Clinical Investigation and The Association of American Physicians. Dr. Weiss' mission as Hematology Chairman at St. Jude Children's Research Hospital is to facilitate cutting edge basic, translational and clinical research, foster faculty development and promote outstanding clinical care for patients with non-malignant blood disorders.

Reappointment of Scientific Members to the Grants Working Group

Grants Working Group Members originally appointed in 2008-11 have terms that are now expiring or just expired. We are seeking the reappointment of the individuals listed in the table below. Their updated biographies follow. In accordance with the rules set forth by Proposition 71, reappointments should be staggered into thirds, each with a 2, 4, or 6-year term.

Proposed Reappointments to GWG

Last	First	Term	Expertise
Barrett	Julia	6	Clinical Regulatory for Small Molecules & Biologics
Breuer	Christopher	6	Pediatric Surgery; Tissue Engineering; Vascular Biology
Broccoli	Vania	4	Genetics & Neural Stem Cell Fate; Hereditory Neural Syndromes; Neurodevelopment
Broeckel	Ulrich	6	Genetics & Cardiovascular Disease; Pharmacogenetics; iPSC Modeling
De Bari	Cosimo	4	Skeletal Repair; Potency Assays; Arthritis
Doherty	Daniel	2	Developmental Pediatrics; Genetics & Hindbrain Malformations
Gelb	Bruce	2	Pediatric Cardiology; Cardiomyopathy; Cardiovascular Genetics: Heart Tranplantation
Griffith	May	2	Regenerative Medicine; Biomaterials; Corneal Combination Products
Hei	Derek	6	Developing Cell & Gene Therapeutics for Clinical Trials; Cell Manufacturing
Kotton	Darrell	4	Stem Cell & Gene Therapy; Lung Developmental Biology; Pulmonary Medicine
Laning	Joseph	2	Cell Banking; Biologics and Cellular Therapeutics Product Development
McCauley	Jacob	2	Genetic Epidemiology; Multiple Sclerosis; Neurodegenerative Disease
Palotie	Aarno	4	Genetics & the Central Nervous System; Migraines; Epilepsy; Schizophrenia; Autism
Sadelain	Michel	6	Gene Therapy; Bone Marrow Transplant; Immunology; Cancer
Vallier	Ludovic	4	Pluripotent Stem Cell Biology; Endoderm; iPSC Modeling of Liver & Metabolic Disease
Wade-Martins	Richard	2	Molecular Mechanisms of Neurodegeneration; Gene Therapy
Williams	Grant	6	Drug Development; Clinical Regulatory; Oncology
Zarbin	Marco	4	Opthamology; Cell Therapy

Julia Barrett, MD, MPH
Julia Barrett has been a Senior Clinical Consultant with Biologics Consulting since 2004. She received her BA in biology from Smith College, her MD from Northwestern University School of Medicine, and a Masters degree in public health (MPH) from George Washington University. Dr. Barrett completed an internship and residency in Internal Medicine at the University of Minnesota, and a fellowship in General Internal Medicine at George Washington University before joining the Food and Drug Administration (FDA) as a senior clinical reviewer. After leaving the FDA, Dr. Barrett initially consulted independently

while practicing internal medicine, however since 2004 she has been a full-time clinical regulatory consultant.

Dr. Barrett was a clinical reviewer at the Center for Biologics Evaluation and Research (CBER), FDA from 1992 to 1997 in the Office of Vaccines Research and Review. While at FDA, she was responsible for providing comprehensive clinical review of Investigational New Drug (IND) applications and Biologics License Applications (BLAs). Dr. Barrett's regulatory expertise, coupled with her clinical experience, provides her with a unique perspective on FDA requirements for the design, preparation and implementation of Phase 1, 2, and 3 clinical protocols, as well as overall clinical development strategy. Dr. Barrett assists her clients with designing and conducting clinical programs for a variety of investigational products (biologics, drugs, combination products) and clinical indications. She has planned and participated in many FDA meetings and is involved in the preparation of FDA submissions, including pre-INDs, INDs, briefing packages, BLAs and New Drug Applications (NDAs).

Christopher Breuer, MD
Christopher Breuer is Associate Professor of Surgery and Director of Tissue Engineering at Yale University School of Medicine. Dr. Breuer received his BA in Biology from College of the Holy Cross and his MD from Brown/Dartmouth. He completed an internship and residency in general surgery, a junior residency in pediatric surgery, a postdoctoral fellowship in surgical research, and was Chief Resident in both general surgery and pediatric surgery. Dr. Breuer is board certified in general surgery and pediatric surgery.

Dr Breuer's research interests focus on the development of improved vascular grafts for use in congenital heart surgery where complications arising from currently used vascular grafts are a leading cause of morbidity and mortality. He runs an NIH funded laboratory investigating the cellular and molecular mechanisms underlying vascular neotissue formation in tissue engineered vascular grafts. He is the principal investigator on the first FDA approved trial investigating the use of tissue engineered vascular grafts in humans. Dr. Breuer's clinical interests include minimally invasive and laparoscopic surgery for neonates, newborns, infants and children; neonatal surgery, including prenatal consultation, fetal intervention and postnatal surgical care; extracorporeal membrane oxygenation (ECMO); esophageal atresia; necrotizing enterocolitis; intestinal atresia; pediatric cardiac surgery; and pediatric thyroid and parathyroid surgery.

Vania Broccoli, PhD
Vania Broccoli is Head of Research Unit in the Division of Neuroscience at the San Raffaele Scientific Institute in Milan, Italy. He earned his BSc in Biological Sciences at the University of Bologna, his PhD in Neuroscience at the University of Padua, and completed a postdoctoral fellowship in Neurogenetics at the Helmholtz Center in Munich, Germany. Dr. Broccoli was Staff Scientist at the Telethon Institute for Genetics and Medicine before moving to the Stem Cell Research Institute at the San Raffaele Scientific Institute as Staff Scientist. He was promoted to Group Leader at the Stem Cell Research Institute before becoming Head of Research Unit.

Since joining the San Raffaele Scientific Institute as a tenure track scientist, Dr. Broccoli has been working in unraveling the molecular mechanisms governing forebrain development and functionally characterizing the transcription factors Arx, Tbr2, Tbr1, FoxG1, MeCP2 involved in neurodevelopmental pathologies in humans. In a long-term collaboration with Prof. J. Rubenstein's lab at the University of California San Francisco, Dr. Broccoli's group showed that Tbr2 is essential for determining intermediate cortical progenitors, providing the first experimental evidences that these neural progenitors are the founders of most cortical neurons. These data illuminated the biological origins of Tbr2 dependent microcephaly in humans. His own research group aims to translate this knowledge in the stem cell research field contributing to develop protocols and methods for generating sub-type specific telencephalic neurons through in vitro differentiation of embryonic and neural stem cells.

Lately, his group employed lineage specific transcription factors for reprogramming cellular identity and generating therapeutic relevant neuronal subtypes from conversion of skin fibroblasts. Initially, he established genetic cell reprogramming for generating iPS cells with the aim to model human diseases like Alzheimer's and Parkinson's disease and atypical Rett syndrome. Then, his group identified a minimal combination of three transcription factors (Mash1, Nurr1 and Lmx1a) able to directly convert mouse and human fibroblasts into functional dopaminergic neurons (Caiazzo et al., 2011). This discovery

allows for the straightforward production of a homogenous source of human functional dopaminergic neurons amenable for a cellular replacement therapy in Parkinson's disease. His lab is currently pursuing new procedures of direct cell reprogramming for generating other neuronal cell types with a therapeutic prospective.

Ulrich Broeckel, MD
Uli Broeckel is Professor of Pediatrics, Adjunct Professor of Medicine and Physiology, Chief of the Section of Genomics Pediatrics, Associate Director of the Children's Research Institute, and Director of the Individualized Medicine Institute at the Medical College of Wisconsin. He earned his MD from the University of Heidelberg and completed a residency and internship in the Department of Internal Medicine II: Cardiology, Nephrology and Pulmonary Medicine at the University of Regensburg. Dr. Broeckel was Research Fellow at the University of Ulm before moving to the Medical College of Wisconsin, initially as a postdoctoral fellow in the Laboratory of Genetics Research, where he rose through the ranks to his current position.

Dr. Broeckel's laboratory specializes in the identification and functional evaluation of genes and their variants involved in cardiovascular and other complex diseases. His research interest include: left ventricular hypertrophy, myocardial infarction, coronary artery disease, end-stage renal disease, and hypertension with projects based on large epidemiological studies in clinical cohorts. In addition, his laboratory performs microarray-based diagnostic tests with an emphasis in pharmacogenomics for both Children's Hospital of Wisconsin (CHW) and St. Jude Children's Research Hospital and runs the Nucleic Acid Extraction Core for investigators at Children's Research Institute. Dr. Broeckel's laboratory is also a part of a groundbreaking multicenter National Heart, Lung, and Blood Institute (NHLBI) initiative to generate patient-derived human induced pluripotent stem cells (hiPSC) for the study of complex disease. This collaboration will result in the high-throughput development of hiPSC-derived cardiomyocytes generated from patients participating in a major hypertension epidemiological study.

Dr. Broeckel is a member of several professional and honorary societies, including the American Heart Association (AHA), the American Association for the Advancement of Science (AAAS), and the American Pediatric Society. His research is supported by grants from the National Institutes of Health (NIH) and he is an experienced reviewer for the NHLBI. Dr. Broeckel has published more than 60 articles throughout his career

Cosimo De Bari, MD PhD FRCP
Cosimo De Bari holds a Chair in Translational Medicine at the University of Aberdeen (UK), where his lab is studying resident stem cells in joint health and disease. He is a clinically active rheumatologist with expertise in musculoskeletal regenerative medicine and arthritis pathophysiology.

Cosimo graduated in Medicine (summa cum laude) from the University of Bari (Italy), where he underwent specialist training in Rheumatology. He obtained his PhD from the University of Leuven (Belgium), where he was the recipient of the 2003 Rotary Young Investigator Award from the Royal Belgian Society for Rheumatology. While at King's College London (UK), he was awarded a Clinician Scientist Fellowship from the Medical Research Council.

At the University of Aberdeen, Cosimo is Founder and Director of the Aberdeen Centre for Arthritis and Musculoskeletal Health, Deputy Director of the Arthritis Research UK Tissue Engineering Centre, and Deputy Director of the EULAR Centre of Excellence in Rheumatology.
Cosimo is editorial board member for several journals, serves on panels of national and international funding bodies including the MRC and CIRM, and sits on international scientific advisory boards.

Dan Doherty, MD, PhD
Dan Doherty is Professor of Pediatrics at the University of Washington in the Divisions of Developmental and Genetic Medicine. His research interests focus on hindbrain malformations as a way to understand human brain development and common disorders such as intellectual disability, autism, ataxic cerebral palsy and even mental health disorders such as schizophrenia.

The hindbrain regulates basic functions (level of consciousness, heart rate, respiratory rate), coordinates balance, limb and eye movements, as well as having possible roles in cognition and emotional regulation. Dr. Doherty's group has used a variety of genetic techniques to identify the genes responsible for

hindbrain malformation disorders such as Joubert, Chudley-McCullough, Poretti Boltshauser syndromes. Identifying the genes responsible for a disorder immediately translates into molecular diagnostic testing, and detailed work on genotype-phenotype correlations improves diagnostic, prognostic and medical management information for patients. In addition, Dr. Doherty's group and his collaborators use the disease genes to dissect the molecular mechanisms underlying normal and abnormal brain development in vitro and in animal models. The human hindbrain is a fascinating system in which to study the role of basic developmental processes (spatially restricted gene expression to define positional information, organizing centers, morphogenetic movements, cell-cell and long-range signaling, cell migration and axon guidance) in human disease.

Dr. Doherty's clinical interests complement the research interests of his group. He cares for children with all types of central nervous system abnormalities including, hindbrain malformations, forebrain malformations, hydrocephalus, agenesis of the corpus callosum, spina bifida, intellectual disability and cerebral palsy. He also provides prenatal counseling to women carrying fetuses with abnormal CNS imaging findings to provide a pediatric perspective on these conditions.

Bruce D. Gelb, MD
Bruce Gelb is the Director and Gogel Family Professor of the Mindich Child Health and Development Institute at the Icahn School of Medicine at Mount Sinai. He is Professor of Pediatrics and of Genetics and Genomic Sciences. Dr. Gelb completed a pediatric residency and pediatric cardiology fellowship at Babies Hospital of Columbia-Presbyterian Medical Center and Texas Children's Hospital at the Baylor College of Medicine, respectively. He joined the faculty at Mount Sinai in 1991 and has remained there since.

Dr. Gelb has developed an extensive program in genomics/gene discovery for congenital heart disease. His group is best known for their work on Noonan syndrome and related disorders (now termed Rasopathies). They have ongoing work in gene discovery, animal modeling, and therapy development. The Gelb group published the first model of cardiovascular disease using human induced pluripotent stem cells, using fibroblasts from patients with Noonan syndrome with multiple lentigine. They continue to use human iPS cells to model cardiovascular disease. In addition to his research, he directs the Cardiovascular Genetics Program at Mount Sinai.

Dr. Gelb has received the E. Mead Johnson Award from the Society for Pediatric Research and the Norman J. Siegel New Member Outstanding Science Award from the American Pediatric Society. He was elected to the American Society of Clinical Investigation and the Institute of Medicine. Dr. Gelb is the President of the American Pediatrics Society and the Treasurer-Elect of the American Society for Human Genetics.

May Griffith, PhD, MBA
May Griffith is a full professor at the Dept. of Ophthalmology, University of Montreal and a researcher at the Maiasonneuve-Rosemont Hospital Research Centre in Montreal, Canada. She holds the Caroline Durand Foundation Research Chair in Cellular Therapy at the University of Montreal, and is Director, Cornea and Anterior Segment of the Quebec Vision Health Research Network. Dr. Griffith received her BS in zoology and human biology, her MSc in zoology, and her PhD in anatomy (developmental cell biology) at the University of Toronto. She received her MBA at the University of Ottawa.

Dr. Griffith's research interests are in biomaterial enhanced or enabled cell-based regeneration. She is best known for leading a multinational, interdisciplinary team that achieved the world's first successful regeneration of the human cornea using biosynthetic implants in a clinical trial. Her team has been developing biomaterials as cell-free implants to promote regeneration of patients' organs by stimulating their endogenous stem or progenitor cells to affect the repair. She and her team have successfully tested the concept in the regeneration of the human cornea in a first-in-human clinical trial of 10 patients. Very recently, they reported a follow-up human clinical trial with six patients diagnosed with being at high risk of rejecting conventional donor corneal transplantation. Because no exogenous cells were introduced, the grafts were immune compatible and the regenerated neo-corneas remained stable. Her biosynthetic and biomimetic materials have also been successfully tested in collaboration with other researchers for use in cartilage and cardiovascular regeneration.

Derek Hei, PhD
Derek Hei is the Senior Vice President of Manufacturing, Quality and Regulatory at BlueRock Therapeutics. Dr Hei has over twenty years of experience developing breakthrough cell and gene therapies from research through clinical trials. He has distinct expertise in current Good Manufacturing Practices (cGMP) compliance, manufacturing, quality control testing and regulatory compliance. At BlueRock, he oversees the entire manufacturing function, which includes developing and producing BlueRock's induced pluripotent stem cell platform, maintaining its state-of-the-art cleanroom facility under cGMP, and leading the regulatory efforts for submitting an investigational new drug application. Dr. Hei also serves as an adjunct professor within the master of science in biotechnology program at UW-Madison where he instructs on aspects of biotherapeutic development including process development, scale-up, analytical methods development and key elements of Chemistry, Manufacturing and Control. Prior to joining BlueRock, Dr. Hei served as vice president of clinical manufacture, quality and regulatory at Cellular Dynamics International, where he oversaw the development and production of its cell therapy products. Prior, Dr. Hei served at Waisman Biomanufacturing, Cerus Corp and Genentech. Dr. Hei obtained his bachelor of science in chemical engineering from the University of Wisconsin-Madison, and his Ph.D. in biochemical engineering from the University of California, Berkeley.

Darrell N. Kotton, MD
Darrell Kotton is the David C. Seldin Professor in the department of medicine and in the department of pathology and laboratory medicine. He conducts basic stem cell research as an NIH-funded principle investigator and is the founding director of the Center for Regenerative Medicine of Boston University and Boston Medical Center. His research focuses on stem cell biology and gene therapy related to lung injury and repair. His laboratory has pioneered induced pluripotent stem cell models of lung development and disease, and a major focus of his team is the engineering of reparative cells, tissues, and drugs for lung regeneration. As an educator, Kotton currently serves as the principal investigator of Boston University's NIH-funded Regenerative Medicine Training Program. His success as a mentor and his self-less approach to Open Source sharing of knowledge and reagents has been recognized through the Senior Research Mentor Award by the Department of Medicine as well as the American Association of Medical Colleges inaugural National Research Resource Sharing Award in 2017.

Joseph Laning, PhD
Joseph Laning is currently the Director of Cell Manufacturing Operations at the Astellas Institute for Regenerative Medicine (AIRM). Dr. Laning received his BS degree in Biology from Boston University and his PhD in Immunology from Harvard University. He is a member of the International Society Cell & Gene Therapy and is the ISCT voting delegate to the USP.

Dr. Laning has spent the past 23 years seeking to translate concepts into products in the fields of wound care, regenerative medicine, and stem cell therapy. He began his post-doctoral career at Organogenesis, Inc. where he developed and implemented pre-clinical investigations and subsequently managed all patient immunology safety testing leading to the approval of a Premarket Approval Application (PMA) of Apligraf™ with the US Food and Drug Administration (FDA). In 2002, he joined ViaCell, Inc. where he served as Director of Therapeutic Development and subsequently Senior Director of Analytical Biology. In these roles he oversaw strategic and operational scientific plans leading to successful approval of both Investigational New Drug (IND) applications and Investigational Device Exemption (IDE) FDA filings and completion of the company's cell therapy clinical trial in allogeneic cord blood stem cell transplantation. He subsequently brought his extensive experience in scientific management and product development to scientific and business consulting opportunities in industry and academics as Founder and Principal of JCLaning BioConsulting.In 2010 he became Senior Director of the Massachusetts Stem Cell Bank and Registry and Research Associate Professor of Molecular Medicine at the University of Massachusetts Medical School. In this role, he oversaw implementation of procedures and quality testing for all stem cell lines and the marketing of more than 30 iPS and ES lines to researchers. In 2013 Dr. Laning joined Provia Laboratories and became Chief Technology Officer leading strategic implementation of their cGMP cell and tissue banking operations.

Dr. Laning's current interests include developing and enhancing human iPS and ES manufacturing methods for delivery of drug substance and drug product for AIRM clinical programs. In addition, scale up of hPSC cultures for commercialization and automation methods to deliver commercial scale cell therapeutics are current priorities.

Jacob L. McCauley, PhD
Jacob McCauley is an Associate Professor of Human Genetics and Pathology at the University of Miami Miller School of Medicine within the Dr. John T. Macdonald Foundation Department of Human Genetics. He is also the Director of the Center for Genome Technology & Biorepository Facility at the John P. Hussman Institute for Human Genomics. Dr. McCauley is a graduate of Bethany College in Bethany, WV (BS, 2000) and Vanderbilt University (PhD, 2005) in Nashville, TN. Following his PhD training in molecular genetics, he completed a postdoctoral fellowship in genetic epidemiology and then took a faculty position at the University of Miami. Dr. McCauley's PhD dissertation focused on understanding the genetic mechanisms involved in autism, with his postdoctoral training focused on the genetic aspects of both Alzheimer's disease and multiple sclerosis.

Dr. McCauley is a genetic epidemiologist with a background comprised of training in both molecular and statistical genetic techniques. His primary interest is to improve the understanding of human disease through disease gene discovery, genomics, and in-depth examination of environmental factors that influence disease outcome. His research focuses on the use of molecular techniques, bioinformatics, and statistical methods to identify genetic variation and to characterize its role in disease susceptibility within a variety of diseases including multiple sclerosis, inflammatory bowel disease (IBD), stroke, and amyotrophic lateral sclerosis (ALS). Dr. McCauley's role as Director of the Center for Genome Technology and Biorepository Facility has multiple administrative responsibilities (e.g. research and development, staffing, budget management). This role has also provided him the opportunity to consult and direct biological sample collections, oversee biological sample tracking, quality control procedures, genotyping, sequencing and genetic analyses involved in large-scale consortia projects. Combined these efforts have aided in a number of discoveries within the genetics of complex disease phenotypes.

In addition to leading his own NIH-funded research projects, Dr. McCauley contributes to a number of other large collaborative research efforts both nationally and internationally. He has been an active member of the International Multiple Sclerosis Genetics Consortium (IMSGC) for more than a decade, the Biorepository Core Director of the Ethnic/Racial Variations of Intracerebral Hemorrhage (ERICH) study led by Dr. Daniel Woo at the University of Cincinnati over the last eight years. He also serves as a Co-PI for Project #2 of the "Clinical Research in ALS & related disorders for Therapeutic Development (CREATE)" project (PI: Dr. Benatar) as part of the NIH's Rare Diseases Clinical Research Consortia and the PI of the Biospecimen and Clinical Data Core of the NIMHD funded Precision Medicine & Health Disparities Collaborative (PI: Dr. Wilkins; Vanderbilt). Finally, he is also a Co-I within the SouthEast Enrollment Center Consortium, a Health Provider Organization (HPO) partner within the NIH *All of Us* Research Program. While his primary research role within each of these various studies is unique, they share a common thread of experience in large-scale NIH-funded collaborative and team science initiatives.

Aarno Palotie, MD, PhD
Aarno Palotie is the research director of the Human Genomics program at FIMM. He is also a faculty member at the Center for Human Genome Research at the Massachusetts General Hospital in Boston and associate member of the Broad Institute of MIT and Harvard. He has a long track record in human disease genetics. He has hold professorships and group leader positions at the University of Helsinki, UCLA, Wellcome Trust Sanger Institute, The Broad Institute of MIT and Harvard and the Massachusetts general hospital. He has also been the director of the Finnish Genome Center and Laboratory of Molecular Genetics in the Helsinki University Hospital. He has served in numerous national and international boards, including the FIMM board.

Dr. Palotie is the Chief Scientific Officer of the large *FinnGen* project (www.finngen.fi) that collects the genome and health record data from 500 000 Finnish participants. The project is contributed by Business Finland (The Finnish innovation fund) and seven international pharma companies with a 59M€ budget. He has also chaired several large international research consortia and as a member or chair in national and international expert panels, including the *International Headache Genetics Consortium* (IHGC) and *Sequencing Initiative Suomi, SISu* (www.sisuproject.fi) the consortium that combines all genome or exome wide sequence data produced from Finnish samples. Aarno Palotie has extensive experience in establishing, running and overseeing infrastructures both in research and clinical settings. In addition to running clinical laboratories and the Finnish Genome Center, he served as the director of Medical Sequencing and a member of the sequencing committee in the Sanger Institute, established and run the

tissue array unit in UCLA and has been a key player in planning the National Genome Strategy and national biobanking strategies in Finland.

Dr. Palotie has also focused on genetics of mental health diseases, particularly schizophrenia and neurodevelopmental diseases. These findings stimulated another large study, the *SUPER*-study with more than 10 000 psychosis cases from Finland. Since the frequency of both schizophrenia and intellectual disability is enriched in Northern Finland,he also initiated the *Northern Finnish Intellectual Disability Cohort* consisting of more than 1000 cases and 1000 family members providing support for the genetic nature of these disabling diseases.

Michel Sadelain, MD, PhD
Michael Sadelain is the Stephen and Barbara Friedman Chair at the Memorial Sloan-Kettering Cancer Center (MSKCC), and is the director of its Gene Transfer and Somatic Cell Engineering Facility. He is also a member of the Department of Medicine and Immunology Program, and the Molecular Pharmacology and Chemistry Program at MSKCC. He received his MD at the University of Paris and PhD at the University of Alberta, followed by a residency at Centre Hospitalier Universitaire Saint-Antoine (Paris) and a fellowship at Massachusetts Institute of Technology.

Dr. Sadelain and his team are actively conducting research at the Gene Transfer and Somatic Cell Engineering Facility, investigating how to control transgene expression in vivo in hematopoietic stem cells, and how to augment immune responses against tumor cells. Until 2007, Dr. Sadelain was co-chair of the program committee and on the board of directors of the American Society of Gene Therapy. He is also a member of the National Heart, Lung, and Blood Institute (NHLBI) Gene Therapy Resource Program review panel, and on the Curriculum Planning Committee at the Sloan-Kettering Institute.

Ludovic Vallier, PhD
Dr. Vallier is Professor of Regenerative Medicine affiliated with the Department of Surgery Cambridge University, Principal Investigator at the Wellcome and MRC Cambridge Stem Cell Institute, Director of the Cambridge Biomedical Research Centre hiPSCs core facility and Head of the “transplant and regenerative medicine” NIHR Biomedical Campus Research campus. He also hold a joint appointment as Senior Group Leader with the Wellcome Sanger Institute. Finally, he is also the co-funder of the biotechnology company DefiniGEN, which is producing hepatocytes and pancreatic cells for drug and toxicology screening. Dr. Vallier received his Magister in Cellular and Molecular Biology and PhD in Cell Biology from ENS Lyon.

Dr. Vallier has developed a strong expertise in human pluripotent stem cells and organoids by discovering key mechanisms controlling their differentiation and selfrenewal. The current objective of Dr. Vallier’s research is to define the molecular mechanisms controlling the specification of the endoderm germ layer from which key organs such as the pancreas and the liver are derived. For that, his group uses human pluripotent stem cells (hESCs and hiPSCs) and organoid as in vitro model of development. The resulting knowledge allows the development of new culture systems to produce liver and pancreatic cells. The resulting cells can then be used to model in vitro metabolic disorders such as non alchohlic liver disease for basic studies and also drug screening. Furthermore, Dr. Vallier’s group explores the potential of liver and pancreatic cells for cell based therapy approach. Overall, his objective is not only to differentiate human pluripotent stem cells or organoid into cell types relevant for clinical applications but also to acquire the knowledge necessary to differentiate any cell types into pancreatic and hepatic progenitors.

Dr. Vallier’s contributions to the stem cell field have generated over 70 original papers, 9 book chapters and 20 reviews on basic aspects of stem cell biology and resulted in numerous invitations to internationally recognized meetings such as the European association for the study of the liver, the European society for gene therapy and Keystones Symposia. Furthermore, he has been awarded a prestigious ERC advanced grant in 2017 to continue his work on liver diseases. Finally, he is recognized as a worldwide expert in hiPSC derivation and differentiation through his activity in the BRC hiPSC core facility and in the UK human induced pluripotent stem cell initiative.

Richard Wade-Martins, PhD
Dr. Wade-Martins heads the Laboratory of Molecular Neurodegeneration at the University of Oxford, focused on better understanding the molecular and genetic mechanisms of neurodegenerative diseases, and is also Director of the Oxford Parkinson's Disease Centre. The Centre joins together ten laboratories

in Oxford and supports a full time staff of fourteen posts focused on understanding the very earliest molecular pathways to pathology in Parkinson's disease. Dr. Wade-Martins studied Natural Sciences (Genetics) at the University of Cambridge, followed by PhD at the Wellcome Trust Centre for Human Genetics, University of Oxford. He then held consecutive Wellcome Trust Research Fellowships at the University of Oxford, Harvard University and then again at Oxford. In 2008, he was appointed to a faculty position at the Department of Physiology, Anatomy and Genetics, University of Oxford, made Associate Professor in 2014 and full Professor in 2015.

Dr. Wade-Martins' career is focused on improving our understanding of the molecular and genetic mechanisms of neurodegenerative diseases with the aim one day of developing novel protective therapies for neurological disorders. In 2004, he returned from Harvard Medical School to the Wellcome Trust Centre for Human Genetics in Oxford and was awarded a Wellcome Trust Research Career Development Fellowship to study the molecular genetics of gene expression from the microtubule associated protein tau (MAPT or tau) genomic locus. His range of interests has expanded and his group now studies the molecular mechanisms of degeneration associated with tau, alpha-synuclein, leucine rich repeat kinase 2 (LRRK2), TDP-43, FUS and C9ORF72 using human post-mortem brain tissue, BAC transgenic mouse lines and in vitro neuronal culture systems. His research includes a major effort to develop iPS cell-derived dopaminergic neuronal culture models to study Parkinson's disease. Dr. Wade-Martins heads the "CNS: Neurodegenerative and neurodysfunctional diseases" theme in the new StemBANCC consortium, a large European Union-funded collaborative study between academia and industry to develop iPS cell models for common diseases, and leads the MRC Dementia Platform UK Stem Cell Network for studying iPSC models of neurodegeneration.

Dr. Wade-Martins has held numerous highly competitive Wellcome Trust Fellowships and is a previous Research Into Ageing UK Young Investigator. He currently sits on the Executive Committee of the Dementia Platform UK and the Strategy Advisory Board for Parkinson's UK. He is Director of the Oxford Parkinson's Disease Centre and a Fellow of Christ Church College, Oxford.

Grant Williams, MD
Grant Williams is a consultant in oncology clinical trial design and regulatory strategy. He received his medical degree and subsequent training in internal medicine, pathology, and medical oncology at the University of Alabama in Birmingham from 1977-1988. For 16 years, from 1989 to 2005, he worked in the Division of Oncology Drug Products at FDA evaluating cancer drug applications, 8 years as a medical reviewer, 5 years as a medical team leader and 3 years as Deputy Director. During his tenure at FDA he contributed to 3 guidance documents, including the Cancer Endpoints Guidance. He subsequently spent three years as Executive Director in oncology clinical development at Novartis and GSK. He has authored numerous papers and book chapters on cancer drug endpoints and oncology drug regulation. In 2008 Dr. Williams formed a consulting company, Williams Cancer Drug Consulting LLC, which provides advice on oncology clinical trial design and regulatory strategy.

Marco Zarbin, MD, PhD, FACS
Marco Zarbin is professor and chair at the Institute of Ophthalmology and Visual Science, and Professor of Neurosciences at Rutgers-New Jersey Medical School. He is also chief of the Department of Ophthalmology at University Hospital, Newark. He received his MD and PhD from The Johns Hopkins University School of Medicine, and completed residency and fellowships in vitreoretinal surgery and retinal vascular disease at The Wilmer Ophthalmological Institute at Johns Hopkins. He is certified with the American Board of Ophthalmology.

Dr. Zarbin specializes in medical and surgical diseases of the retina, vitreous, and macula, with research interests in retinal cell transplantation and growth factors. His research is focused on developing new surgical treatments for age-related macular degeneration, the leading cause of blindness in persons above age 55. His clinical interests are limited to medical and surgical diseases of the retina and vitreous, with special expertise in age-related macular degeneration, trauma management, complex retinal detachment (including retinopathy of prematurity), and surgery of the macula (including macular holes, subretinal hemorrhage, and subretinal neovascularization).

Dr. Zarbin is Editor-in-Chief of *Translational Vision Science and Technology*, and is a member of the editorial board of *Ophthalmology*, *Ophthalmology-Retina*, *Asia Pacific Journal of Ophthalmology*, *Journal*

of Vitreoretinal Diseases. He is vice chair of the scientific advisory board of the Foundation Fighting Blindness, a member of the board of Governors (ex officio) of the New Jersey Academy of Ophthalmology, and a member of the National Advisory Council of the National Institutes of Health. In addition, he is a member of the American Ophthalmological Society, Academia Ophthalmologica Internationalis, the Club Jules Gonin, the Retina Society, the Macula Society, and the American Society of Retina Specialists. Dr. Zarbin is past president of the board of trustees of the Association of University Professors of Ophthalmology. Dr. Zarbin has co-authored 222 peer-reviewed scientific publications and 104 book chapters, and has co-edited one book on age-related macular degeneration, 2 books on diabetic retinopathy, and 1 book on cell-based therapy for degenerative retinal disease.