

Nominations for Appointment to the Grants Working Group (GWG)

NEW APPOINTMENTS

Maria Basil, MD, PhD, Assistant Professor of Medicine, Perelman School of Medicine, University of Pennsylvania

Referral: Maria Basil was identified by CIRM Review Team.

<u>Expertise Relevance to CIRM GWG:</u> Maria Basil's expertise in pulmonary repair and regeneration will be invaluable in reviewing Clinical and Discovery program applications.

<u>Prior Service in CIRM Reviews:</u> Maria Basil has participated in Discovery and Preclinical program reviews.

Dr. Maria Basil an Assistant Professor of Medicine at the Perelman School of Medicine at the University of Pennsylvania. She is a member of the Cardiovascular Institute (CVI) and the Lung Biology Institute (LBI) at Penn. Her current research builds upon her long-term pursuit of understanding complex cellular relationships and cell fates in development and regeneration. The Basil laboratory focuses on understanding how the human lung undergoes injury, repair, and regeneration and how this is altered in disease by utilizing cutting-edge technologies and novel approaches to interrogate human lung biology.

During her post-doctoral work, Dr. Basil delineated the characteristics and functions of novel progenitor cell populations that comprise the human respiratory bronchioles. She identified a human-specific lung progenitor cell termed Respiratory Airway Secretory (RAS) cells and showed that RAS cell progenitor function is altered in human lung disease. Her work presently focuses on this region, working to understand the development of the supportive niche for this population as well as the signaling pathways that regulate its function in both health and disease. Dr. Basil is supported by the NHLBI, the American Cancer Society, and the Burroughs Wellcome Fund.

Sanjay Bidichandani, MBBS, PhD, CHF Claire Gordon Duncan Chair in Genetics, David L. Boren Professor of Pediatrics, University of Oklahoma College of Medicine

Referral: Sanjay Bidichandani was identified by CIRM Review Team.

<u>Expertise Relevance to CIRM GWG:</u> Sanjay Bidichandani's expertise in gene therapy for inherited neurologic disorders will be invaluable in reviewing Clinical and Preclinical program applications.

<u>Prior Service in CIRM Reviews:</u> Sanjay Bidichandani has participated in Clinical program reviews.

Sanjay Bidichandani received his medical degree from Pune University (India), a PhD in Medical Genetics from Glasgow University (Scotland), and postdoctoral training in neurogenetics at Baylor College of Medicine (Houston, TX), where he also graduated from the Master Teacher Fellowship program. He is currently the Claire Gordon Duncan Chair in Pediatric Genetics, and David L. Boren Professor of Pediatrics at the University of Oklahoma College of Medicine. His research, which has been funded by NIH, DoD, MDA and FARA, is focused on the precise genetic and epigenetic defect in Friedreich ataxia. He currently serves on the Board of Directors of the Friedreich Ataxia Research Alliance, and on the Research Advisory Committee of the Muscular Dystrophy Association. He previously served as Assistant Dean (pre-clinical curriculum) in the College of Medicine, Vice President for Research at the Muscular Dystrophy Association, and as a member / chair of the Programmatic Panel of the Congressionally Directed Medical Research Programs' Neurofibromatosis Research Program (US Department of Defense). He has served on various national and international grant review panels. He has mentored several PhD students and postdoctoral fellows. He is a four-time recipient of the Aesculapian award for teaching excellence, and a recipient of the Stanton L. Young Master Teacher Award by the University of Oklahoma College of Medicine.

Manfred Boehm, MD, Senior Investigator, Branch Chief, National Heart Lung and Blood Institute, National Institutes of Health

Referral: Manfred Boehm was identified by CIRM Review Team.

<u>Expertise Relevance to CIRM GWG:</u> Manfred Boehm's expertise in cardiovascular regenerative medicine will be invaluable in reviewing Discovery and Preclinical program applications.

Prior Service in CIRM Reviews: NA

Dr. Manfred Boehm is a prominent researcher at the National Heart, Lung, and Blood Institute (NHLBI), renowned for his pioneering work in rare inherited vascular diseases. His research centers on using patient-specific cell model systems to uncover the molecular mechanisms of these diseases and explore stem cell potential for tissue repair and regeneration. Dr. Boehm's innovative approach has led to breakthroughs in disease modeling via induced pluripotent stem cell (iPSC) technology, enabling differentiation into various mesodermal-derived cell types and the creation of complex organoids that simulate vascular and inflammatory processes (Wimmer et. al. Nature. 2019 Jan;565(7740)). These efforts aim to develop therapeutic strategies for conditions such as ischemic heart disease, leading to new disease identifications and treatments for cardiovascular conditions (Lin et. al.2024, Cell Stem Cell, 31(7)). Additionally, Dr. Boehm has organized significant events like the Symposium on Cardiovascular Regenerative Medicine (2004-2017), fostering collaboration and knowledge-sharing among experts in the field. Under Dr. Boehm's leadership, the Vascular Translational Program at NHLBI uses personalized medicine approaches to address clinical challenges in rare vascular diseases. By employing high-risk research strategies and cutting-edge technologies, the program identifies genetic variants and discovers novel disease mechanisms, driving the development of targeted therapies. It enrolls patients with rare monogenic diseases featuring vascular

pathomechanisms, integrating clinical/genomic evaluation with patient-specific in vitro and in vivo models. The program excels in characterizing disease phenotypes and developing genomics-focused research to identify molecular mechanisms, ultimately aiming to unravel complex vascular pathophysiology and assess therapeutic efficacy through first-in-human clinical trials. By leveraging personalized medicine and collaborating with NHLBI, NIH, and external labs, Dr. Boehm utilizes advanced technologies to discover genetic variants, elucidate disease-causing mechanisms, and develop new therapeutic options. His work significantly broadens knowledge in vascular biology and addresses unmet clinical needs for patients with rare diseases, leading to innovative disease-specific protocols and treatment strategies in vascular medicine.

Alisha Bouge, MS,

Assistant Teaching Professor, Director of Practicum, School of Pharmacy, Biomedical Regulatory Affairs Master of Science (BRAMS), University of Washington

Referral: Alisha Bouge was identified by CIRM Review Team.

<u>Expertise Relevance to CIRM GWG:</u> Alisha Bouge's expertise in regulatory affairs and clinical trials will be invaluable in reviewing Clinical program applications.

Prior Service in CIRM Reviews: NA

Alisha R. Bouge is a regulatory affairs leader with over 17 years of experience in biopharmaceuticals, higher education, and clinical research. She holds an MS in Biomedical Regulatory Affairs and is US RAC-certified, with a proven track record in developing and executing regulatory strategies for US FDA submissions, including INDs, IDEs, and Orphan Drug Designations.

As the Executive Director of Regulatory Affairs at Exegenesis Bio, Alisha led critical Phase 1 milestones and built a high-performing regulatory team. Her expertise spans gene therapy, rare diseases, and early-phase clinical trials, with hands-on involvement in trial execution, regulatory submissions, and cross-functional leadership. Previously, at Stanford University, she directed regulatory and quality assurance for stem cell and gene therapy trials, expanding the team and streamlining FDA submissions. As an Assistant Teaching Professor at the University of Washington, she shapes the next generation of regulatory professionals through mentorship and curriculum development.

Bruce Burnett, PhD,

Chief, Office of Regulatory Affairs, Division of Allergy, Immunology and Transplantation, NIAID, National Institutes of Health

Referral: Bruce Burnett was identified by CIRM Review Team.

<u>Expertise Relevance to CIRM GWG:</u> Bruce Burnett's expertise in regulatory affairs and clinical trials will be invaluable in reviewing Clinical program applications.

Prior Service in CIRM Reviews: NA

Dr. Burnett has over 25 years of experience in multiple aspects of clinical drug development, biologics, and medical devices. His regulatory experience includes 9 years in the biotech industry and 16 years in an academic setting. Dr. Burnett received his Ph.D. in organic chemistry/biochemistry from the Massachusetts Institute of Technology under Nobel Laureate Dr. Har Gobind Khorana, followed by an NIH Postdoctoral Fellowship at Harvard University/Harvard Medical School. Dr. Burnett held various positions within the biopharmaceutical industry, first at NEN/DuPont, followed by research and quality positions at Serono, Genetics Institute, and Biogen. At Genetics Institute, Dr. Burnett managed a team of 25 analysts with five direct reports, responsible for all in-process, release, and stability testing for all clinical recombinant drug substances and drug products. Specific areas of responsibility include immunoassay, biochemical, structural characterization, chemistry, and biological assays. The group was responsible for validating all QC release and characterization assays. At Biogen, Dr. Burnett led a bioanalytical group of 30 analysts with four direct reports responsible for commercial and investigational drug substance and drug product release testing, product characterization, and stability testing, as well as testing samples from nonclinical, clinical, and immunogenicity studies. Dr. Burnett transitioned to the Regulatory Affairs department in 1998, where he eventually became the regulatory lead for the submission and eventual approval of natalizumab (Tysabri) for multiple sclerosis. Dr. Burnett oversaw the preparation and approval of all sections of the BLA, utilizing the Common Technical Document structure, to facilitate simultaneous submissions in both the US and Europe.

In 2004, Dr. Burnett took a position as the head of regulatory affairs and quality at a small biotech company in North Carolina, eventually becoming Vice President. In 2007, Dr. Burnett took a position at Duke University School of Medicine to develop a Regulatory Affairs function as part of the newly granted Clinical and Translational Science Award. In 2016, Dr. Burnett served on an IPA as the Director of the newly formed Office of Research Support and Compliance (ORSC) within the NIH Intramural Research Program, reporting to the Deputy Director. After one year, he transitioned to support ORSC as a special advisor to the Deputy Director on issues relating to cGMP with respect to cell therapy manufacturing, quality, and regulatory affairs until June 2024. In August 2024, Dr. Burnett assumed a full-time position within the Division of Allergy, Immunology, and Transplantation at the NIAID, NIH, as the Chief of the Office of Regulatory Affairs.

Barry J. Byrne, M.D., Ph.D. Professor and Associate Chair Department of Pediatrics, University of Florida, College of Medicine

Referral: Barry Byrne was identified by CIRM Review Team.

<u>Expertise Relevance to CIRM GWG:</u> Barry Byrne's expertise in pediatrics rare disease and gene therapy will be invaluable in reviewing Clinical Preclinical program applications.

Prior Service in CIRM Reviews: NA

Dr. Barry Byrne is a clinician scientist who is studying a variety of rare diseases with the specific goal of developing therapies for inherited muscle disease. As a pediatric cardiologist, his focus is on conditions that lead to skeletal muscle weakness and abnormalities in heart and respiratory function. His group has made significant contributions to the understanding and

treatment of Pompe disease, a type of neuromuscular disease due to glycogen storage in motor units. The research team has been developing new therapies using AAV-mediated gene therapy to restore cardiac and skeletal muscle function in DMD, Friedreich's ataxia, Pompe, and other inherited neuromuscular diseases. His group at the Powell Center has also established a series of new methods for large-scale AAV clinical manufacturing. The work is supported by several NIH, industry and foundation awards.

Dr. Byrne is the Associate Chair of Pediatrics and Director of the University of Florida Powell Gene Therapy Center and Child Health Research Institute. He obtained his B.S. degree from Denison University; M.D. and Ph.D. from the University of Illinois; and he completed his Pediatrics residency and cardiology fellowship as well as post-doctoral training in Biological Chemistry at the Johns Hopkins Hospital. Following his early career at Hopkins, he joined the University of Florida and is now the Earl and Christy Powell University Chair in Genetics. In addition to his academic appointments, he serves as the Chief Medical Advisor of the Muscular Dystrophy Association and as a member of the MDA Board of Directors.

Kirsten Cowley, Vice President of Clinical Operations for Rare Disease at REGENXBIO

Referral: Kirsten Cowley was identified by CIRM Review Team.

<u>Expertise Relevance to CIRM GWG:</u> Kirsten Cowley's expertise in clinical operations will be invaluable in reviewing Clinical program applications.

Prior Service in CIRM Reviews: NA

Kirsten Cowley is Vice President of Clinical Operations for Rare Disease at REGENXBIO, where she leads gene therapy programs from INDs through BLA. A proven leader with over 30 years of experience in medical product development, Kirsten brings expertise in clinical operations and regulatory compliance, data management, and process optimization. She has successfully driven programs across Hematology, Oncology, Vaccines, and Rare Diseases, with a focus on gene therapy for the past 13 years.

At REGENXBIO, she has advanced multiple rare disease programs to pivotal regulatory milestones, streamlined operations to accelerate timelines, and built high-performing teams. Known for strategic execution and cross-functional leadership, Kirsten excels in guiding critical decisions and optimizing resources across global trials. Kirsten is passionate about developing treatment options for rare diseases and champions patient-centric development to ensure that every program is designed with the needs of patients and families at the core.

Beverly L. Davidson, PhD,

Katherine A High, Chair in Cell and Gene Therapy, Director, Raymond G Perelman Center for Cellular and Molecular Therapeutics, Chief Scientific Strategy Officer, Children's Hospital of Philadelphia, Professor, Pathology and Laboratory Medicine, University of Pennsylvania

Referral: Beverly Davidson was identified by CIRM Review Team.

<u>Expertise Relevance to CIRM GWG:</u> Beverly Davidson's expertise in neurogenerative disorders will be invaluable in reviewing Clinical and Preclinical program applications.

Prior Service in CIRM Reviews: NA

Beverly L. Davidson, PhD is the Director, Raymond G. Perelman Center for Cellular and Molecular Therapeutics, Chief Scientific Strategy Officer, and holds the Katherine A. High Chair in Cell and Gene Therapy at Children's Hospital of Philadelphia. She is Professor of Pathology and Laboratory Medicine, Perelman School of Medicine, University of Pennsylvania. She received her Ph.D. in Biological Chemistry from University of Michigan.

The Davidson lab is focused on genetic diseases that affect the brain, including how mutant gene products contribute to disease, and why certain brain regions are more susceptible. The team employs advanced molecular methods, sequencing and imaging modalities in animal models, and uses a variety of molecular tools to test various hypotheses. The lab is also engaged in the development of next generation therapeutics for inherited disorders, including the engineering of novel gene therapy vector capsids and cargo to approach tissue and cell type specific treatments. She is a founder of Spark Therapeutics, Spirovant Biosciences and more recently Latus Bio.

Recent honors include the Hereditary Disease Foundation's Leslie Gehry Brenner Prize for Innovation in Science, the Dr. John W. Schut Research Achievement Award from the National Ataxia Foundation, and elections into the American Academy of Arts and Sciences and the National Academy of Medicine. She is past president of the American Society of Gene and Cell Therapy, the largest international association of gene and cell therapy research, and recent recipient of the Transformative Research Award from the Hereditary Research Foundation.

David DiGiusto, PhD, Independent Consultant

Referral: David DiGiusto was identified by Clinical and Review Teams.

<u>Expertise Relevance to CIRM GWG:</u> David DiGiusto's expertise in CMC regulatory affairs will be invaluable in reviewing Clinical program applications.

Prior Service in CIRM Reviews: David DiGiusto has participated in Clinical program reviews.

David DiGiusto PhD is formally trained as an immunologist and stem cell biologist with over 21 years of academic and 12 years of industrial experience in translational medicine with a focus on blood and pluripotent stem cells as therapeutic agents. David is an established leader in the field of cell and gene therapy and has made significant contributions to product development and manufacturing for more than a dozen clinical assets through Phase I/II clinical trials. He has been responsible for the design, build and operation of over a dozen GMP - compliant biologics manufacturing facilities in both academic and industrial applications. His responsibilities have included creation and oversight of process development, manufacturing, quality systems, and regulatory affairs infrastructure. Under his direction, plasmid DNA, CAR T-cells, regulatory T-cells, engineered stem cell grafts, iPSC-derived and gene modified hematopoietic stem cell and pancreatic islet products have been developed, manufactured and used in clinical

investigations. Dr. DiGiusto is the former the North American Vice President for ISCT (2016-2018), former member of the NIH recombinant DNA advisory committee (RAC) and an independent biotechnology consultant (DiGiusto Consulting LLC. Est. 1985). He received his undergraduate and Ph.D. degrees from the University of Colorado.

David DiGiusto is currently an independent biotechnology consultant. Previously, David held the position of SVP, Cell & Gene Therapies Preclinical & Clinical Mfg - CMC, while working at Vertex Pharmaceuticals Incorporated. David held this role for 13 months (February 2020 - March 2021). Before this, David held the position of Chief Technical Officer, while working at Semma Therapeutics. David occupied this position for 19 months (August 2018 - March 2020). Previously, David held the position of Executive Director - Stem Cell and Cellular Therapeutics Operations, while working at Stanford Health Care. David held this role for 3 years (March 2015 - June 2018). Before this, David held the position of Research Professor - Virology, while working at City of Hope. David occupied this position for 17 years (August 1998 - February 2015). David completed a Ph.D. •Microbiology and Immunology degree from the University of Colorado Anschutz Medical Campus, in 1985 - 1990. Additionally, David received a BA •Molecular in Cellular and developmental Biology, in 1978 - 1982, from the University of Colorado Boulder.

Mauro Giacca, MD, PhD,

Head of the School of Cardiovascular and Metabolic Medicine & Sciences at King's College London, Director of the UK MRC/BHF Centre of Excellence for Advanced Cardiac Therapies (REACT)

Referral: Mauro Giacca was identified by CIRM Review Team.

<u>Expertise Relevance to CIRM GWG:</u> Mauro Giacca's expertise in gene therapy will be invaluable in reviewing Clinical and Preclinical program applications.

Prior Service in CIRM Reviews: NA

Professor Mauro Giacca, MD PhD, is the Head of the School of Cardiovascular and Metabolic Medicine Sciences at King's College London and the Director of the UK MRC/BHF Centre of Excellence for Advanced Cardiac Therapies (REACT). He moved to the United Kingdom in 2019, after having served as the Director-General of the International Centre for Genetic Engineering and Biotechnology (ICGEB), a research organization in the United Nations system. Prof Giacca is considered an expert in cardiovascular gene therapy using AAV vectors and the development of biologics and RNA therapies for cardiac repair and regeneration. He has pioneered the concept that cardiac regeneration can be obtained through the stimulation of endogenous cardiomyocyte proliferation using small RNAs. He is the founder of Forcefield Therapeutics and Heqet Therapeutics, two start ups that develop advanced therapies for myocardial infarction and heart failure. His research is funded internationally, including three consecutive ERC Advanced Investigator grants from the European Commission, two consecutive Fondation Leducq Transatlantic Networks of Excellence grants, and two consecutive Programme grants from the British Heart Foundation. He is regularly invited to present his research in seminars and meetings internationally.

He has published over 450 papers peer-reviewed international journals, including Nature, Science, Cell, Nature Med, Nature Cardiovascular Research, Science Transl Med, Circulation and others. He sits in the Scientific Advisory Board of several research centres and biotech companies internationally and is a reviewer for various granting programs, including those from the European Commission (including the European Research Council) and numerous international funding agencies.

Alison Goate, D.Phil.,

Jean C. & James W. Crystal Professor and Chair, Director, Ronald M. Loeb Center for Alzheimer's Disease, Dept. of Genetics & Genomic Sciences, Icahn Genomics Institute, Icahn School of Medicine at Mount Sinai

Referral: Alison Goate was identified by CIRM Review Team.

<u>Expertise Relevance to CIRM GWG:</u> Alison Goate's expertise in neurodegenerative disorders will be invaluable in reviewing Discovery and Preclinical program applications.

Prior Service in CIRM Reviews: NA

Dr. Alison Goate is the Jean C and James W. Crystal Professor and Chair of the Dept. of Genetics and Genomic Sciences at the Icahn School of Medicine at Mount Sinai (ISMMS). She has worked on the genetics of neurodegenerative diseases including Alzheimer's disease (AD) and Frontotemporal Dementia (FTD) since 1987 and is the founding director of the Ronald M. Loeb Center for Alzheimer's disease at ISMMS. She reported the first mutation to cause familial Alzheimer's disease. Dr. Goate is also a leader in the study of late onset AD genetics using both GWAS and sequencing approaches. Her team demonstrated the enrichment of AD risk variants in microglial enhancers, regulatory elements that control gene expression in immune cells of the brain. She is now building upon these insights using genome-editing in induced pluripotent stem cells to understand the molecular mechanisms of disease and to develop novel therapeutics. Dr. Goate has received many awards for her research including the Potamkin Award from the American Academy of Neurology, the Khalid Iqbal Lifetime Achievement Award from the Alzheimer's Association, the MetLife Award and the Rainwater Prize for Innovation in Neurodegeneration. She was elected a fellow of AAAS in 2012 and a fellow of the National Academy of Medicine in 2016.

Steven Gray, PhD,

Helen J and Robert S Strauss Distinguished Professor in Pediatric Neurology, University of Texas Southwestern Medical Center

Referral: Steven Gray was identified by CIRM Review Team.

<u>Expertise Relevance to CIRM GWG:</u> Steven Gray's expertise in gene therapy will be invaluable in reviewing Clinical and Preclinical program applications.

Prior Service in CIRM Reviews: NA

Dr. Steven Gray received a B.S. degree with honors from Auburn University followed by a Ph.D. in molecular biology from Vanderbilt University and postdoctoral training at the UNC Chapel Hill

Gene Therapy Center. He is currently a Professor in the Department of Pediatrics at the University of Texas Southwestern Medical Center. Dr. Gray is the co-director of the UTSW Gene Therapy Program and director of the UTSW Viral Vector Facility. He maintains affiliations with the Department of Molecular Biology, the Department of Neurology, and the Eugene McDermott Center for Human Growth and Development at UT Southwestern.

Dr. Gray's core expertise is in AAV gene therapy vector engineering, followed by optimizing approaches to deliver a gene to the nervous system. His major focus is in AAV vector development to develop vectors tailored to serve specific clinical and research applications involving the nervous system. These include the development of novel AAV capsids amenable to widespread CNS gene transfer. As AAV-based platform gene transfer technologies have been developed to achieve global, efficient, and in some cases cell-type specific CNS gene delivery, his research focus has also included preclinical studies to apply these reagents toward the development of treatments for neurological diseases. Currently these have resulted in approved and ongoing human clinical trials to test gene therapies for the following disorders: Rett Syndrome, Giant Axonal Neuropathy (GAN), Tay-Sachs, Sandhoff, Spastic Paraplegia Type 50, Charcot Marie Tooth disease type 4J, and Batten Diseases (CLN1, CLN5, and CLN7).

Dr. Gray has published over 100 peer-reviewed papers in journals such as *New England Journal of Medicine, Molecular Therapy, Brain, Journal of Clinical Investigations, Nature Biotechnology, Nature Medicine,* and *The Proceedings of the National Academy of Sciences.* He also has over 25 pending or awarded patents. His research is funded by the National Institute for Neurological Disorders and Stroke, as well as numerous large and small research foundations and industry partners. Dr. Gray was recognized with the American Society of Gene and Cell Therapy's Outstanding Young Investigator Award in 2019, the 2016 Healthcare Hero award by the Triangle Business Journal, and his work on GAN was featured in a story by the CBS National Evening News in 2015.

Nancy Guo, PhD, SUNY Empire Innovation Professor at Binghamton University

Referral: Nancy Guo was identified by CIRM Review Team.

<u>Expertise Relevance to CIRM GWG:</u> Nancy Guo's expertise in AI precision medicine will be invaluable in reviewing Discovery and Preclinical program applications.

Prior Service in CIRM Reviews: NA

Nancy Guo, Ph.D., is a SUNY Empire Innovation Professor at Binghamton University. Dr. Guo is a Fulbright US Scholar for Brazil on biomarker discovery. Dr. Guo is experienced in leading foundation Al-based multidisciplinary research as PI of two NIH R01s and two NSF grants. She obtained over \$45.5 M in federal funding as PI/PD to develop technology and infrastructure to advance precision medicine. She has 62 peer-reviewed journal publications with more than 4,062 citations. Her research has generated 14 patents on cancer drugs and molecular diagnostic assays with FDA "Novel Technology" status. Software products developed by her team have more than 46,000 visits. As the founding director of the Biomedical Informatics Resources Core of West Virginia Clinical & Translational Science Institute from 2009-2017, she led state-wide informatics initiatives and enhanced multi-state collaboration. Through her current

NSF PFI-RP project, she fosters academic-industry partnerships for clinical commercialization of AI-based cancer treatment selection and drug development. She mentored 48 postdocs, MDs, and graduate students to strive as next-generation researchers, tenured faculty, entrepreneurs as PI of NSF and NCI SBIRs, and healthcare professionals recognized with nine national awards. She served as Chair of grant review panels of lung cancer research for the DOD and NCI.

Jasmina Kapetanovic, MA, BMBCh Oxon, MRCOphth, MSc, PhD, FRCOphth University of Oxford and Oxford Eye Hospital, UK

Referral: Jasmina Kapetanovic was identified by CIRM Review Team.

<u>Expertise Relevance to CIRM GWG:</u> Jasmina Kapetanovic's expertise in eye vision disorders and clinical trials will be invaluable in reviewing Clinical program applications.

<u>Prior Service in CIRM Reviews:</u> Jasmina Kapetanovic has participated in Clinical program reviews.

Dr. Kapetanovic is an MRC Clinician Scientist and a Consultant Ophthalmic Surgeon at Nuffield Laboratory of Ophthalmology where she leads The Interventional Genetics and Robotics for Retinal Disease Research Group. Kapetanovic group focuses on developing groundbreaking therapies for inherited retinal degenerations, the leading cause of blindness in the working population, as well as therapies for more common forms of blindness, the age-related macular degeneration (AMD), retinal vein occlusions and glaucoma.

Kapetanovic received her undergraduate (MA in Physiological Sciences, Balliol College) and medical degrees at the University of Oxford. She was awarded the UK's first NIHR integrated clinical academic fellowship programme in ophthalmology and has since awarded Masters in Investigative Ophthalmology and Visual Science (First Class) and a PhD in Medicine (gene therapy and optogenetics) via the MRC Clinical Research Training Fellowship at the University of Manchester, UK. Her PhD in optogenetic vision restoration was awarded the prestigious Russell Medal for the best UK paper in vision research and several outstanding awards including the MRC Centenary Award and Retinitis Pigmentosa Fighting Blindness Award. As a Keeler scholar at the University of California, Berkeley, she conducted a post-doctoral fellowship into vector biology for therapeutic delivery to retina. She completed the specialist training and was admitted as a fellow (FRCOphth) into the UK Royal College of Ophthalmologists, has published widely and won numerous awards during her early career as an academic ophthalmologist. She is now a Consultant Ophthalmic Surgeon specialised in cataract and vitreoretinal surgery, and as a Clinician Scientist she is currently working on developing innovative surgical techniques including robot-assisted retinal surgery for future applications in genetic therapies. In addition, Kapetanovic has expertise in clinical trial design and has been involved in leading several first in human gene therapy and robotic eye surgery trials including those for Choroideremia, RPGR and USH2A-related retinal dystrophy and age-related macular degeneration.

Rohit N. Kulkarni, MD, PhD, Professor of Medicine, Harvard Medical School and Associate Director of the Diabetes Research Center at Joslin Referral: Rohit Kulkarni was identified by CIRM Review Team.

<u>Expertise Relevance to CIRM GWG:</u> Rohit Kulkarni's expertise in endocrine disorders and diabetes will be invaluable in reviewing Discovery program applications.

Prior Service in CIRM Reviews: NA

Rohit N. Kulkarni received his MD and PhD degrees from Bangalore Medical College, St. John's Medical College and Royal Post-Graduate Medical School in London, UK. During his Endocrine Fellowship training at Hammersmith Hospital, he obtained his PhD (Biochemistry; University of London). He completed a Post-Doc Fellowship at the Joslin Diabetes Center in Boston, USA, and has been on the Joslin Diabetes Center and Harvard Medical School Faculty since 1999. Dr. Kulkarni holds the Diabetes and Wellness Foundation Endowed Chair, and is Section Head of Islet Cell Biology at Joslin Diabetes, Professor of Medicine, Harvard Medical School and Associate Director of the Diabetes Research Center at Joslin. He is an Associate Member of the Broad Institute at MIT/Harvard and Principal Faculty at Harvard Stem Cell Institute. He has held multiple leadership positions and has mentored over 70 trainees who have gone on to hold independent positions in academia and industry. He is recognized internationally for outstanding research in diabetes and metabolism by the Ernst Oppenheimer Award (Endocrine Society), the Albert Renold Prize (European Association for the Study of Diabetes; EASD), the Paul E. Lacy Medal, the Julio V. Santiago MD Memorial Lecture, and Dean's Distinguished Lecture. He is an elected member of the American Society for Clinical Investigation, the Association of American Physicians and American Association for the Advancement of Science. He has published over 200 peer-reviewed manuscripts, and his laboratory is interested in investigating mRNA modifications, stem cell biology and organ crosstalk in the long-term goal of finding cures for diabetes and obesity.

Michael A. Laflamme, MD, PhD,

Robert R. McEwen Chair in Cardiac Regenerative Medicine, Senior Scientist, McEwen Stem Institute, University Health Network (UHN), Staff Pathologist, Laboratory Medicine & Pathology Program, UHN, Canada Research Chair in Cardiovascular Regenerative Medicine, Professor, Department of Laboratory Medicine & Pathobiology, University of Toronto

Referral: Michael Laflamme was identified by CIRM Review Team.

<u>Expertise Relevance to CIRM GWG:</u> Michael Laflamme's expertise in gene therapy will be invaluable in reviewing Clinical and Preclinical program applications.

Prior Service in CIRM Reviews: NA

Dr. Michael Laflamme is the Robert McEwen Chair in Cardiac Regenerative Medicine, Canada Research Chair (Tier 1) in Cardiovascular Regenerative Medicine, Senior Scientist in the McEwen Stem Cell Institute, Staff Pathologist in the Laboratory Medicine Program at the University Health Network (UHN), and Professor of Laboratory Medicine & Pathobiology at the University of Toronto. After obtaining an undergraduate degree in Physics at Georgetown University, Dr. Laflamme completed the Medical Scientist (MD/PhD) Training Program at Emory

University, where he studied the regulation of calcium homeostasis by beta-adrenergic signaling in adult ventricular cardiomyocytes. After residency in Anatomic Pathology and subspecialty training in cardiovascular pathology at the University of Washington Medical Center, he completed a postdoctoral fellowship in the laboratory of Dr. Charles Murry, investigating the role of exogenous and endogenous stem cells in myocardial repair.

Dr. Laflamme was faculty at the University of Washington from 2006 to 2015, when he relocated to UHN and the University of Toronto. His independent research program has focused on the development of novel cardiac cell therapies based on human pluripotent stem cells (hPSCs). His laboratory has made a number of important contributions in this area including efficient protocols to guide hPSCs into cardiomyocytes, proof-of-concept transplantation studies with hPSC-derived cardiomyocytes in small- and large-animal models, and the first direct demonstration that hPSC-derived cardiomyocytes can become electrically integrated and activate synchronously with host myocardium in injured hearts. Dr. Laflamme has been the recipient of honors including the Society for Cardiovascular Pathology Young Investigator Award, the American Society of Gene & Cell Therapy Outstanding New Investigator Award, and the UHN Inventor of the Year. He has organized and led a number of large, multi-investigator projects, including team grants supported by the Ontario Institute for Regenerative Medicine, the Stem Cell Network, and the Government of Canada's New Frontiers in Research Fund. Dr. Laflamme also practices cardiovascular and autopsy pathology and is a founding investigator of BlueRock Therapeutics.

Piemonti Lorenzo, MD,

Director of the Diabetes Research Institute and Chief of the Department of Regenerative Medicine and Transplantation, San Raffaele Scientific Institute in Milan, Italy

Referral: Piemonti Lorenzo was identified by CIRM Review Team.

<u>Expertise Relevance to CIRM GWG:</u> Piemonti Lorenzo's expertise in diabetes product development will be invaluable in reviewing Clinical and Preclinical program applications.

Prior Service in CIRM Reviews: NA

At the San Raffaele Scientific Institute in Milan, Italy, Lorenzo Piemonti serves as the Director of the Diabetes Research Institute and Chief of the Department of Regenerative Medicine and Transplantation. In addition, he leads the Beta Cell Biology Unit and directs the Human Islet Processing Facility. He previously coordinated the European Consortium for Islet Transplantation, which has been instrumental in providing human beta cell products for both research and clinical applications at HSR, within Italy, and across Europe since 2000.

He holds the position of Professor in Endocrinology at the University "Vita Salute San Raffaele" in Milan and served as a Visiting Professor at Vrije Universiteit of Brussel from 2016 to 2022. Piemonti's academic expertise spans diabetes and pancreatology, with specific interests in the interplay between pathophysiology, innovative therapeutics, and precision medicine. His research focuses on advancing the understanding and treatment of diabetes, particularly through beta cell replacement therapies, immune tolerance induction strategies, stem cell research, and dendritic cell biology. He has a specific interest in beta cell differentiation and maturation processes, the development of biomaterials and scaffolds to support islet

transplantation, and strategies to protect beta cells from immune-mediated damage in autoimmune diabetes. Additionally, his work addresses immune tolerance in the context of transplantation, exploring mechanisms to prevent graft rejection while minimizing systemic immunosuppression. His laboratory has also investigated the role of innate immune responses and their influence on adaptive immunity in diabetes and transplantation, with a focus on dendritic cells and their tolerogenic potential.

In the broader realm of regenerative medicine, Piemonti's research investigates the differentiation and reprogramming of stem cells, aiming to generate functional insulin-producing cells for therapeutic purposes. He has also contributed to the development of bioengineered pancreas and organoids as platforms for studying diabetes pathogenesis and testing novel therapies. Beyond diabetes, he has conducted significant work in pancreatic oncology, particularly the interactions between cancer cells, stromal cells, and the tumor microenvironment. His interests include leveraging regenerative medicine tools and immune-modulating therapies to explore innovative strategies for treating pancreatic cancer. As a physician-scientist, Piemonti is deeply committed to translational research, aiming to bridge the gap between cutting-edge scientific discoveries and their practical application in clinical settings. This includes designing clinical trials for cell replacement therapies and immune interventions and integrating high-dimensional data to tailor precision medicine approaches for diabetes and related disorders.

Foteini Mourkioti, PhD, Associate Professor of Orthopaedic Surgery, Perelman School of Medicine, University of Pennsylvania

Referral: Foteini Mourkioti was identified by CIRM Review Team.

<u>Expertise Relevance to CIRM GWG:</u> Foteini Mourkioti's expertise in skeletal muscle regeneration, stem cells and muscle disorders and aging will be invaluable in reviewing Discovery program applications.

Prior Service in CIRM Reviews: NA

Dr. Mourkioti is an Associate Professor in the Departments of Orthopaedic Surgery and Cell and Developmental Biology at the University of Pennsylvania, Perelman School of Medicine. She is also the co-Director of the Penn Institute for Regenerative Medicine, Musculoskeletal Program. Dr. Foteini Mourkioti has a profound interest in skeletal muscle and cardiac disease mechanisms. Her lab aims to unravel 3 fundamental aspects of cell biology: how stem cells sense their tissue environment, including mechanosensitivity; how they communicate this information; and what are the nuclear mechanisms by which telomeric proteins sustain genome integrity *in vivo*. Dr. Mourkioti is driven by her scientific desire to understand the role of progenitor cells in muscle diseases, and she has a strong background in muscle biology, mechanobiology skills and substantial stem cell expertise from her nearly 20 years working in the muscle field, including experience with cellular mechanisms in several muscle diseases and genetic mouse models.

Her lab is pioneering the role of telomere biology in skeletal muscles as well as the development of state-of-the-art imaging approaches to directly observe and dissect the mechanisms that

regulate tissue regeneration *in vivo*. By visualizing muscle stem cells in their endogenous environment, she discovered that they exhibit morphological heterogeneity, displaying a variability of axon-like cellular protrusions. Her studies have changed the classical conception of dormancy of adult resident stem cells and provided a paradigm for quiescence and activation during physiological and pathological conditions, with an emphasis on muscle chronic injuries, such as Duchenne Muscular Dystrophy. In addition, her research demonstrated that in another disease, called fibrodysplasia ossificans progressive (FOP), which is characterized by misguided repair and formation of aberrant bone within muscles, there is improper communication of resident muscle stem cells (MuSCs) with another muscle progenitor cell, called fibro-adipogenic progenitor (FAPs). Overall, the long-term goal of the Mourkioti lab is to leverage these insights into future therapies that target aberrant cell-cell communication and signaling dysregulation in affecting tissue-tissue interactions during regeneration.

Kiran Musunuru, MD, PhD, MPH, ML, MRA, Barry J. Gertz Professor for Translational Medicine in the Perelman School of Medicine at the University of Pennsylvania

Referral: Kiran Musunuru was identified by Preclinical and Review Teams.

<u>Expertise Relevance to CIRM GWG:</u> Kiran Musunuru's expertise in cardiovascular and metabolic diseases will be invaluable in reviewing Clinical and Preclinical program applications.

Prior Service in CIRM Reviews: NA

An actively practicing cardiologist and committed teacher, Kiran Musunuru, MD, PhD, MPH, ML, MRA, is the Barry J. Gertz Professor for Translational Medicine in the Perelman School of Medicine at the University of Pennsylvania. His research focuses on the development of novel gene editing therapies for the treatment of cardiovascular and metabolic diseases. 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, Harvard University's Fannie Cox Prize for Excellence in Science Teaching, and the University of Pennsylvania's Jane M. Glick Graduate Student Teaching Award. 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 cofounder and Senior Scientific Advisor of Verve Therapeutics.

Karen Nichols, ESQ, President/Owner, Nichols Enterprises LLC

Referral: Karen Nichols was identified by CIRM Review Team.

<u>Expertise Relevance to CIRM GWG:</u> Karen Nichols' expertise in clinical operations for cell and gene therapy products will be invaluable in reviewing Clinical program applications.

Prior Service in CIRM Reviews: NA

Karen Nichols is an accomplished regulatory and quality executive with over 40 years of experience leading global regulatory strategy, quality systems, and compliance programs across the biotechnology and pharmaceutical sectors, with preclinical, clinical and commercial expertise spanning cell and gene therapy, enzyme replacement therapies, and antibody-drug conjugates to support the advancement of innovative therapies from early development through licensure. She has prepared and submitted INDs, IMPDs/CTAs, BLAs, and MAAs. She has participated in and led multiple regulatory interactions with agencies including the FDA, EMA, MHRA, and Health Canada to include preIND, Type B, Type C, Type D, Scientific Advice, End of Phase, pre-MAA, preBLA meetings, PRIME, RMAT, OOPD as well as responded to multiple information requests to support CD+34 enriched cell therapy, mobilization peptide, differentiated cell therapy-combination and gene-edited somatic cell product submissions. She has established quality and regulatory systems and provided strategic regulatory and quality operational review and oversight for facility construction and qualification, preclinical studies, clinical peptide and CGT/implantable device manufacturing and qualification, and commercial ERT manufacturing.

Folshade Otegbeye, MD, MPH, Associate Professor, Dept of Medicine, University of Washington

Referral: Folshade Otegbeye was identified by CIRM Review Team.

<u>Expertise Relevance to CIRM GWG:</u> Folshade Otegbeye's expertise in hematologic conditions including cancer will be invaluable in reviewing Clinical program applications.

Prior Service in CIRM Reviews: NA

Folashade "Shade" Otegbeye, MBChB MPH. Dr. Shade Otegbeye is an Attending Physician and Associate Professor at Fred Hutchinson Cancer Center (Fred Hutch) and the University of Washington School of Medicine, Division of Medical Oncology, in Seattle Washington. Her clinical practice is in the care of patients receiving hematopoietic cell transplant and cellular immunotherapy. She is the Facility Director of the Therapeutic Products Program at Fred Hutch where she oversees the technology transfer and manufacture of immune effector cell products and other biologic agents for administration in a variety of clinical trials. She has research expertise in cellular immunotherapy, cell therapy product development and clinical manufacturing, cGMP operations, quality and regulation. Dr. Otegbeye holds the Fleischauer Family Endowed Chair in Cell and Gene Therapy Translation at Fred Hutch.

Dr. Otegbeye received her medical degree (MBChB) from the Obafemi Awolowo University in Ile-Ife, Nigeria, then a Master of Public Health from the Harvard School of Public Health. She subsequently completed an Internal Medicine residency with Bridgeport Hospital Yale New Haven Health, followed by Hematology and Oncology fellowship at Case Western Reserve University. She translated preclinical research and cell manufacturing experience from her fellowship into her faculty position at Case Comprehensive Cancer Center (Cleveland Ohio) between 2016 and 2021, when she also served as the Medical Director of the Cell Processing Facility. During this period, she led the development of an off-the-shelf natural killer cell platform and the evaluation of these products in a first-in-human trial of these cell products in colon cancer and acute myeloid leukemia patients.

Eirini Papapetrou, MD, PhD,

Professor of Oncological Sciences, Professor of Medicine, Hematology and Medical Oncology, Director, Center for Advancement of Blood Cancer Therapies, Co-Director, Stem Cell Engineering Core, Icahn School of Medicine at Mt Sinai

Referral: Eirini Papapetrou was identified by CIRM Review Team.

<u>Expertise Relevance to CIRM GWG:</u> Eirini Papapetrou's expertise in cancer and cell therapies will be invaluable in reviewing Preclinical program applications.

Prior Service in CIRM Reviews: NA

Eirini Papapetrou, MD, PhD, is a Professor of Oncological Sciences, Hematology and Medical Oncology and founding Director of the Center for Advancement of Blood Cancer Therapies at the Icahn School of Medicine at Mount Sinai (ISMMS). She obtained MD and PhD degrees at the University of Patras in Greece. During her postdoctoral studies at Memorial Sloan-Kettering Cancer Center, she pioneered the generation of patient-derived induced pluripotent stem cells (iPSCs) and their use in disease modeling and regenerative medicine and developed cutting edge technologies for the genetic engineering of iPSCs and hematopoietic stem cells for cell and gene therapy. As an independent investigator, first at the University of Washington and, since 2014, at ISMMS, she performed groundbreaking research that established human iPSCs as models of myeloid malignancies - specifically acute myeloid leukemia (AML) and myelodysplastic syndrome (MDS) -, as well as premalignant conditions - inherited bone marrow failure syndromes (IBMFS) and clonal hematopoiesis (CH). By combining patient cell reprogramming with CRISPR/Cas9-mediated precise gene editing, the Papapetrou laboratory develops models of myeloid leukemias and preleukemic blood disorders and exploits the unique capabilities they offer: the ability to perform genotype-to-phenotype studies and to study the oncogenic mechanisms of driver mutations in a faithful cellular and genomic environment; to obtain large numbers of homogeneous cells for multi-omics analyses, genetic and small molecule screens; and to validate findings through functional assays in isogenic conditions. With these models and strategies Dr Papapetrou's research program seeks to uncover novel disease mechanisms and identify and validate new therapeutic targets.

Dr. Papapetrou is the recipient of several awards, including the American Society of Gene and Cell Therapy Outstanding New Investigator Award, Damon Runyon-Rachleff Innovation Award, Pershing Square Sohn Prize, AACR-MPM, and others, and is an elected member of the American Society for Clinical Investigation. Since 2023, she serves as inaugural Director of the Center for Advancement of Blood Cancer Therapies at ISSMS, a new Center that aims to expand the use of human models of leukemia and other blood diseases and bring together basic, translational and clinical researchers to accelerate the development of new therapies for hematologic malignancies.

Stephanie Protze, PhD, Scientist, McEwen Stem Cell Institute, University Health Network Assistant Professor, Department of Molecular Genetics, University of Toronto

Referral: Stephanie Protze was identified by CIRM Review Team.

<u>Expertise Relevance to CIRM GWG:</u> Stephanie Protze's expertise in cardiovascular disease will be invaluable in reviewing Discovery program applications.

Prior Service in CIRM Reviews: NA

Dr. Stephanie Protze, PhD, is a cardiovascular stem cell scientist and holds the McEwen Stem Cell Institute Chair in Cardiac Regenerative Medicine. Her research program applies cutting-edge stem cell biology, molecular biology, and electrophysiology methods to study the development and diseases of the human heart with a special focus on the cardiac conduction system that regulates the heart rhythm. Dr. Protze's team strives to pursue translational research and aims to develop cell therapies using stem cell-derived biological pacemakers as novel treatments for patients with heart rhythm disorders.

Dr. Protze received her PhD in Cell Biology and Biomedicine from the International Max Planck Research School at the University of Dresden, Germany. For her postdoctoral fellowship, she moved to Toronto, Canada to join Dr. Gordon Keller's laboratory, where she specialized in stem cell research. In 2018, Dr. Protze was appointed as a Principal Investigator at the McEwen Stem Cell Institute at the University Health Network and Assistant Professor in the Department of Molecular Genetics at the University of Toronto. Her research has been published in leading journals such as Nature Biotechnology, Nature Communication, Cell, and Cell Stem Cell.

Scott Schliebner, MPH, Vice President and Global Head, Drug Development Consulting, Novotech

Referral: Scott Schliebner was identified by CIRM Review Team.

<u>Expertise Relevance to CIRM GWG:</u> Scott Schliebner's expertise in drug development and clinical operations will be invaluable in reviewing Clinical program applications.

Prior Service in CIRM Reviews: NA

Scott is a strategic and innovative drug development executive with 30 years' experience across the biopharma, contract research organization (CRO), and non-profit sectors. Passionate about ensuring the patient voice is incorporated into drug development, Scott is focused on patient-focused innovations that reduce the burden of clinical trial participation. With a particular focus on rare diseases, Scott has built partnerships and collaborations that have led to the acceleration of drug development for rare disease patients across the globe. Scott regularly speaks on topics such as patient-focused drug development, innovation in clinical trials, and novel collaborations to accelerate drug development. He has served on the Board of several non-profit organizations and serves as a Strategic Advisor for numerous early-stage firms in the biopharma services sector. Scott received MPH degree in Biostatistics from the University of Utah School of Medicine and completed a Graduate Research Fellowship at The National Institutes of Health/NINDS.

REAPPOINTMENTS

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

Proposed Reappointments to GWG

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Last	First	Term	Years	Expertise
Bailey	Alex	3	6	Regulatory Affairs & Product Development
Barker	Roger	3	6	Cell & Gene Therapies for Neurodegenerative Disease
Kuritzkes	Daniel	3	6	HIV Therapeutics; HIV Persistence & Eradication; Antiretroviral Drug Resistance
Lund	Troy	2	4	HSC Transplantation for Inherited Metabolic Disorders
Mehta	Samir	3	6	Tissue Engineering for Cartilage & Bone Repair
Parenteau	Nancy	3	6	Biotechnology; Biological Products; Drug Development

Alex Bailey, PhD

Dr. Alex Bailey is the co-founder and CEO of Tern Therapeutics, a clinical-stage gene therapy company with a focus on rare disease. He was previously Head of Early Program & Portfolio Development at REGENXBIO Inc, a gene therapy company developing treatments for retinal, metabolic and neurodegenerative diseases. During his tenure at REGENXBIO, he also served as Product Development Lead and Global Regulatory Lead for numerous preclinical and clinical-stage programs supporting all phases of development. Prior to REGENXBIO Inc. Alex spent over five years at the FDA Center for Biologics Evaluation and Research (CBER), initially as a Pharmacology/Toxicology Reviewer and subsequently as the Team Leader in the Pharmacology/Toxicology Branch in the Office of Cellular, Tissue and Gene Therapies (OCTGT; subsequently reorganized into OTP) where he provided scientific and regulatory oversight for CBER-regulated products across broad therapeutic areas (rare disease, regenerative medicine, oncology, other) and technology platforms (cell and gene therapies, immunotherapies, therapeutic cancer vaccines, tissue engineering-based products, combination products, other). Dr. Bailey received his PhD in Biomedical Engineering from the University of Virginia and conducted post-doctoral research at the Fred Hutchinson Cancer Research Center in Seattle, WA.

Dr. Bailey has served as a GWG member for 8 years. He has reviewed for Clinical and Translational program awards.

Roger A. Barker, BA, MBBS, MRCP, PhD

Dr. Barker is Professor of Clinical Neuroscience at the University of Cambridge and an Honorary Consultant Neurologist at the Addenbrooke's Hospital in Cambridge UK. Dr. Barker did his undergraduate training at Oxford University and his clinical training at St Thomas' Hospital in London. He then completed his general medical training in London and moved to Cambridge in 1991 to undertake a PhD at the University on neural grafting in PD with Professor

Stephen Dunnett and James Fawcett. He then completed his specialist training in neurology i and returned to Cambridge as an MRC Clinician Scientist Fellowship and took up his current position in 2000.

Dr. Barker combines clinical research in Huntington's (HD) and Parkinson's disease (PD) with more fundamental research in the laboratory on better therapies for these conditions, including cell-based approaches. The clinical research concentrates on defining the spectrum of deficits in these disorders and the heterogeneity of these diseases and the basis for this. He also is an active neurologist who sees patients with general neurological problems in clinics and in the hospital, in addition to which he runs specialist clinics for patients with HD and PD.

Dr. Barker has published over 600 papers and sits on the editorial boards of many journals and is Co-Editor in Chief of the Journal of Neurology and an Associate Editor of the Journal of Parkinson's Disease. He is the new Chair of Neurodegeneration at LifeArc and was a former Director of the ISSCR. He has undertaken a number of gene and cell therapy trials in PD and HD as well as been involved in trials of drug repurposing and ASO treatments in HD.

Dr. Barker has served as a GWG member for 12 years. He has reviewed for Clinical and Translational program awards.

Daniel R. Kuritzkes, MD

Daniel R. Kuritzkes, MD received his BS and MS degrees in Molecular Biophysics and Biochemistry from Yale University, and his MD from Harvard Medical School. He completed his clinical and research training in internal medicine and infectious diseases at Massachusetts General Hospital and was a visiting scientist at the Whitehead Institute for Biomedical Research before joining the faculty at the University of Colorado Health Sciences Center. Dr. Kuritzkes returned to Harvard Medical School in 2002, where he is now the Harriet Ryan Albee Professor of Medicine and Chief, Division of Infectious Diseases at Brigham and Women's Hospital.

Dr. Kuritzkes has published extensively on antiretroviral therapy and drug resistance in HIV-1 infection. He has chaired several multicenter studies of HIV therapy and previously chaired the AIDS Clinical Trials Group. He has served on numerous NIH committees, including as a member of the NIH Office of AIDS Research Advisory Council. He is a former member of the Department of Health and Human Services panel on guidelines for antiretroviral therapy and a past Chair of the HIV Medicine Association Board of Directors. He has been a member of several editorial boards and served for two decades as an Associate Editor of the Journal of Infectious Diseases. His research interests focus on HIV therapeutics, antiretroviral drug resistance, HIV eradication and more recently, COVID-19.

Dr. Kuritzkes has served as a GWG member for 12 years. He has reviewed for Clinical program awards.

Troy Lund, MD, PhD

Dr. Troy Lund is a Tenured Professor in the Department of Pediatrics at the University of Minnesota where he serves as a physician faculty member and pediatric bone marrow transplant specialist. Dr. Lund is interested in the use of blood and marrow transplantation primarily for patients with inherited metabolic disorders, like adrenoleukodystrophy (ALD) and

Hurler syndrome. His work both in his laboratory and with his patients has created many new approaches to treatment, which will ultimately make transplant safer and more effective.

Dr. Lund's research focuses on improving the outcomes for all patients undergoing blood and marrow transplantation by increasing the speed at which hematopoietic stem cells reconstitute the immune system after transplant. He also works to increase our understanding the pathophysiological processes underlying inherited metabolic diseases. One area Dr. Lund is exploring is how an autoimmune reaction may trigger the cerebral form of adrenoleukodystrophy (cALD), the most serious form of ALD. This study represents the largest screening for immune-reactivity in cALD ever performed, and further research could help identify ALD patients with immune-reactivity prior to the onset of cALD.

Dr. Lund earned his PhD in cancer biology at the University of South Florida and his medical degree at the University of Minnesota Medical School where he also completed a residency and fellowship in hematology/oncology and blood and marrow transplantation. He has received several awards during his time at the University of Minnesota including a Team Science Award. Basic Science Paper of the Year, and Butterfly Award for Outstanding Medical Research.

Dr. Lund has served as a GWG member for 6 years. He has reviewed for Clinical and Translational program awards.

Samir Mehta, MD

Samir Mehta, MD is an Associate Professor in the Department of Orthopaedic Surgery and Chief of the Orthopaedic Trauma & Fracture Service at the Perelman School of Medicine at the University of Pennsylvania. Samir did his Orthopaedic Surgery residency at the University of Pennsylvania where he was a research fellow and was also awarded the DeForest Willard Award. Having developed an interest in Orthopaedic Traumatology, Samir furthered his education as an orthopaedic trauma fellow at Harborview Medical Center in Seattle, Washington. Upon completion of his orthopaedic trauma fellowship, Samir was awarded the AO John Border Memorial European Fellowship Award and continued his education at the University of Saarland in Homburg, Germany. Samir served as clinical research advisor of the Biedermann Lab for Orthopaedic Research at the University of Pennsylvania and is currently the medical director for clinical research for the Department of Orthopaedic Surgery. Samir also served as associate Program Director for nearly a decade. As a result of a busy clinical practice, he has developed a particular interest in pelvic and acetabular fractures, repair of nonunions and malunions, and reconstruction of peri-articular fractures.

Samir is also actively involved with the American Academy of Orthopaedic Surgery, the American Orthopaedic Association, the Orthopaedic Trauma Association, and the AO Foundation. He has been awarded the prestigious American Orthopaedic Association-North American Traveling Fellowship, the AO Howard Rosen Teaching Award, and also has served as an AAOS Health Policy Fellow on Capitol Hill. His research interests include non-unions, open fractures, traumatic articular cartilage injury, and fracture healing. He has participated in over two-hundred peer review publications, has multiple current funded studies, and has presented on numerous occasions nationally and internationally. He has been awarded several grants from agencies such as the DOD, PCORI, and NIH. He has been actively involved in multiple research projects examining graduate medical education and was a faculty member for ten years on the AAOS Course for Orthopaedic Educators. In addition, Samir enjoys running, cooking, and traveling.

Dr. Mehta has served as a GWG member for 12 years. He has reviewed for Clinical and Translational program awards.

Nancy L. Parenteau, PhD

Dr. Parenteau is the co-founder of BioConsultants whose focus is on analyzing, communicating, developing and supporting industry practices that can reduce the development risk of novel technology and increase the efficiency and effectiveness of translational efforts in biotech. Nancy was previously the co-founder, President and CSO of Verik Bio, Inc. focused on novel target identification for engineered T cell-based immunotherapies targeting the regeneration-capable cells of solid tumors. She is the former Senior Vice President ad CSO of Organogenesis and was co-founder and CEO of a biotech start-up, Amaranth Bio, Inc.

She brings many years of hands-on research and management experience to the challenge of bioscience development, having built and managed top cross-disciplinary teams of engineers, biochemists, cell and molecular biologists, and immunologists. Nancy has operated at technology's cutting edge throughout her career from her academic beginnings in monoclonal antibody research in its earliest days while at Georgetown, developing a novel ELISA assay commercialized while a Harvard postdoc, to pioneering the first living product to achieve clinical trial success and US FDA approval.

Dr. Parenteau was named as an International Fellow of Tissue Engineering and Regenerative Medicine by the Tissue Engineering Regenerative Medicine International Society (TERMIS) "in recognition of [her] formative role in shaping the tissue engineering and regenerative medicine field." She has served on the Chemical Sciences Roundtable of the National Research Council. Dr. Parenteau has served as a scientific and strategic advisor to leading companies involved in regenerative medicine and bioactive products and has served as a reviewer for translational projects for the NIH SBIR program. She is an author on biotech leadership, an executive coach, and a strategic advisor to executive management teams, specializing in developing highly skilled, adaptive, and resilient leaders in trailblazing biotech, biopharma, and medical devices.

Dr. Parenteau has served as a GWG member for 12 years. He has reviewed for Clinical and Translational program awards.