



## **Nominations for Reappointment to the Grants Working Group (GWG)**

### **Marcela Maus, MD, PhD**

Marcela Maus is currently an Associate Professor at Harvard Medical School, the Paula O'Keefe Chair in Oncology and Director of Cellular Immunotherapy at Massachusetts General Hospital (MGH) Cancer Center, an Attending Physician in the Hematopoietic Cell Transplant and Cell Therapy division of Oncology at MGH. She is an Associate Member of the Broad Institute of Harvard and MIT, and an Associate Member of the Ragon Institute of MGH, MIT, and Harvard.

Dr. Maus is internationally known for her work as a translational physician-scientist in the field of immunology, particularly as it relates to T-cell immunotherapies and cellular therapies in the treatment of cancer. Her laboratory focuses on the biology of human T cell activation, co-stimulation, and memory, and on the application of human T cell therapies to human disease, including forward and reverse translation of engineered T cell therapies in early-phase clinical trials. She has authored over 100 papers indexed in pubmed and holds three NIH R01 grants and several Investigational New Drug applications (IND's).

Dr. Maus holds an undergraduate degree from Massachusetts Institute of Technology (MIT) and graduate degrees (M.D., Ph.D.) from University of Pennsylvania. Dr. Maus trained in internal medicine at University of Pennsylvania and in hematology and medical oncology at Memorial Sloan Kettering Cancer Center, is board-certified in these three disciplines, and practices medical oncology. She also serves on several scientific and clinical advisory boards for the biotechnology industry as well as external academic medical centers.

### **J. Joseph Melenhorst, PhD**

Jan Joseph (Jos) Melenhorst is a translational T cell immunologist whose career evolved from elucidating the immunobiology of autoimmune disorders to developing a better understanding of T cell responses across HLA barriers and anti-tumor immunity by TCR-guided and chimeric antigen-reprogrammed T cells. He obtained his PhD at the University of Leiden, Netherlands (Department of Hematology) on the immune etiology of Aplastic Anemia. In 1998 he moved to the National Institutes of Health in Bethesda, Maryland, where he did his research - first as a postdoc, later as a staff scientist - on the immunobiology of marrow failure syndromes, leukemic disorders, and allogeneic stem cell transplantation. In 2012 he was recruited by Dr. Carl June to the University of Pennsylvania, first as Deputy Director of their clinical manufacturing (cGMP) facility. After a year he was promoted to Director of Product Development & Correlative Sciences. In this role, he was at the cusp of the first ever CAR T cell therapy approved by FDA: Kymriah. Further, Dr. Melenhorst led to the development of correlative assay pipeline for the first triple CRISPR/Cas9 genome edited, TCR tumor-redirected T cell product in the USA, published early January of 2020 in Science magazine. In 2020 he was promoted to full Professor at the Department of Pathology & Laboratory Medicine where he now fully focuses his effort on the translational sciences of immunogene therapies. His laboratory's goals are to enhance our understanding and improvement of the anti-tumor efficacy and safety of adoptively transferred chimeric antigen receptor-modified T cells through correlative, mechanistic, and functional genomics approaches.

### **William H. Peranteau, MD**

William Hughes Peranteau is an attending surgeon in the Division of Pediatric General, Thoracic and Fetal Surgery at Children's Hospital of Philadelphia (CHOP) and an assistant professor of surgery at the Perelman School of Medicine at the University of Pennsylvania. Dr. Peranteau earned his bachelor's degree in molecular biology from Princeton University, and his medical degree from the University of Pennsylvania. He completed his residency in general surgery at Brigham and Women's Hospital, Boston, Mass., and a fellowship in pediatric general surgery at CHOP. He also served as a research fellow with CHOP for four years, investigating the role of in utero bone marrow and stem cell transplantation for the management of congenital hematologic disorders such as sickle cell disease.

His research focuses are the study of *in utero* gene therapy, including *in utero* gene editing, and *in utero* hematopoietic cell transplantation. Dr. Peranteau has led some of the initial studies of *in utero* CRISPR-mediated gene editing including prenatal base editing to treat metabolic liver and lung diseases in animal models. Dr. Peranteau has published extensively in high impact, pediatric, and surgical journals. He is board certified in pediatric surgery and general surgery.

**John J. Strouse, PhD**

John Strouse is an Associate Professor of Medicine and Pediatrics and Director of the Adult Sickle Cell Program at Duke University. His research has focused on the epidemiology, risk factors, and prevention of the pulmonary and central nervous system complications of sickle cell disease and includes retrospective and prospective cohort studies and clinical trials. He received his PhD in clinical investigation from the Johns Hopkins Bloomberg School of Public Health for a series of studies to identify predictors of cognitive function in children with sickle cell disease. This work has expanded to the evaluation of the interaction between environment and disease in both children and adults with sickle cell disease. Other research interests include functional assessment of older adults with sickle cell disease and the evaluation of interventions to improve quality of and access to care.

Dr. Strouse serves on the American Society of Hematology Sickle Cell Pain Guideline Panel and is co-chair of the American Society of Hematology Healthcare Professional Education and Training Work Group. He is co-chair of the observational study monitoring board for the National Heart Lung and Blood Sickle Cell Disease Studies and the PhenX Sickle Cell Disease Research and Scientific Panel. He is vice-president of the Sickle Cell Adult Provider Network and a member of the Governor’s North Carolina Council on Sickle Cell Syndrome as well as Chair of the Medical Research Committee. He cares for children and adults with non-malignant blood disorders with a focus on sickle cell disease and other hemoglobinopathies.

**Alan Sugar, MD**

Alan Sugar is a Professor in the department of Ophthalmology and Visual Sciences at the University of Michigan Kellogg Eye Center. Dr. Sugar received his BA in Zoology, MD from the School of Medicine and MS in Clinical Research Design and Statistics from the School of Public Health at the University of Michigan. He completed an ophthalmology residency at Washington University in St. Louis and a fellowship in corneal disease and surgery at the University of Florida. He was on the faculty of Mt. Sinai School of Medicine in New York and has been on the faculty of the University of Michigan School of Medicine since 1979, where he became a full professor in 1984.

Dr. Sugar has been involved in clinical research on corneal transplantation, genetic features of Fuchs dystrophy, and new treatments for severely dry eyes. He is also involved with eye research through his work on the research committees of the Eye Bank Association of America and the Michigan Eye Bank. Dr. Sugar belongs to more than 20 professional societies and has published over 225 papers. He has also written or co-written 53 book chapters on ophthalmology and was Editor-in-Chief of the journal *Cornea* from 2012-2019. He has chaired the Institutional Review Board (IRB/MED) of the University of Michigan Medical School since 2011.

**Reappointment of Scientific Members to the Grants Working Group**

We are seeking the reappointment of the individuals listed in the table below. Their updated biographies follow.

**Proposed Third Term Reappointments to GWG**

Last	First	Term	Expertise
Kerr	Doug	6	Drug Development for Neurologic Disease & Injury; Motor Neuron Disease; Neural Injury
Wagner	John	6	Lympho-Hematopoietic Disorders; Bone Marrow Transplantation; Cord Blood

**Doug Kerr, MD**

Doug Kerr is Executive Vice President and Head of Preclinical Research & Clinical Development at Generation Bio. Doug served as global development team lead and vice president for neurology at Shire from 2015-2017 where he was responsible for the development of the rare neuroscience programs including the lysosomal

storage disorders, neurodegeneration, and gene therapy candidates. Doug served as senior director of corporate strategy and portfolio management at Biogen, where he led the development effort for Alzheimer's disease, amyotrophic lateral sclerosis (ALS) and spinal muscular atrophy (SMA). Doug led the development of Spinraza™, now approved for SMA. Doug was on the faculty of The Johns Hopkins School of Medicine as associate professor of neurology with joint appointments in the Department of Molecular Microbiology and Immunology and Cellular and Molecular Medicine, and ran a lab that investigated fundamental aspects of motor neuron/axon biology, provided direct patient care and ran clinical trials, mainly spinal cord and neuromuscular disorders. Doug has participated on the boards and scientific advisory boards of several non-profit organizations, including the Transverse Myelitis Association, CureSMA and the ALS Association.

Doug received his medical degree from Jefferson Medical College, as well as his doctorate in biochemistry and molecular biology. Doug obtained his Master of Business Administration, with a specialization in entrepreneurship and finance, as well as his Bachelor of Science degree in molecular biology from Princeton University.

**John E. Wagner, MD**

John Wagner is a Professor in the Department of Pediatrics, Division of Blood and Marrow Transplant & Cellular Therapy. He is the Founding Director of the new Institute of Cell, Gene and Immunotherapeutics at the University of Minnesota. Co-Director of the Center for Translational Medicine, and holds two endowed chairs — Children's Cancer Research Fund/Hageboeck Family Chair in Childhood Cancer Research, and the University of Minnesota McKnight-Presidential Chair.

Dr. Wagner's research is focused on the development of novel cell therapies for treatment of life threatening diseases. Examples include the development of regulatory T cells that could be used in the treatment of autoimmunity, organ graft rejection as well as graft-versus-host disease, thymic progenitors to repair damaged immune systems, cardiac myoblasts to reverse heart failure, skeletal myoblasts to repair or replace dystrophic muscle fibers in muscular dystrophy, and expansion of the blood-forming stem cell to speed blood and marrow recovery after high doses of chemotherapy and radiation. Dr. Wagner is best known for his pioneering work on the use of placental/cord blood as a source of stem cells for transplantation – a procedure that has now been performed in more than 50,000 patients worldwide.