Nominations for Appointment and Reappointment to the Grants Working Group (GWG)

New ICOC Patient Advocate Appointment to Grants Working Group

Dan Bernal
Patient Advocate, HIV/AIDS

Reappointment of ICOC Patient Advocate Members to Grants Working Group

Last	First	Term	ICOC Role
Higgins	David	6	Patient Advocate, Parkinson's Disease
Rowlett	Al	6	Patient Advocate, Mental Health
Winokur	Diane	6	Patient Advocate, ALS

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. 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 Second Term Reappointments to GWG

Last	First	Term	Expertise
Dere	Will	6	Clinical Development; Endocrine, Bone, & General
Parmar	Malin	6	Neurodegeneration & Parkinson's disease

Will Dere, MD, FACP

Will Dere has extensive experience in clinical research during his 25 year stint in the biopharmaceutical industry. The experience was complemented by substantive regulatory and drug safety experience during that time. At the University of Utah, Dr. Dere serves as the Associate Vice President for Research of Health Sciences, Co-Director of the Utah Center for Clinical and Translational Science, and Co-Director of the Center for Genomic Medicine. He is Professor of Internal Medicine, and the B. Lue and Hope S. Bettilyon Presidential Endowed Professor for Diabetes Research.

While at Eli Lilly from 1989 to 2003, he conducted several clinical pharmacology Phase 1 studies with nasal insulin, several oral antibiotics, and PTH 1-34. He later served as executive director of the clinical pharmacology and the therapeutic areas charged with developing appropriate biomarkers, and proof-of-concept trials in several therapeutic areas. During this time, he supervised the clinical teams responsible for the development of the medicines duloxetine and atomoxetine. His phase 2 and 3 experiences were focused in endocrinology. He led the phase 2 and 3 programs for raloxifene, both in postmenopausal osteoporosis and breast cancer prevention, and PTH 1-34 in postmenopausal osteoporosis and glucocorticoid-induced osteoporosis. Hallmarks of these programs were the large fracture outcomes trials for raloxifene (MORE) published in JAMA and PTH 1-34, published in the NEJM. From 2003 to 2014 at Amgen, Dr. Dere led the development programs of multiple medicines including denosumab marketed for both postmenopausal osteoporosis and prevention of deleterious skeletal outcomes from metastatic cancers; romiplostim for immune thrombocytopenic purpura; panitumumab for colorectal cancer; and cinacalcet for secondary hyperparathyroidism in end-stage renal disease. These programs involved extensive evaluation in both phase 2 and phase 3 clinical trials which occurred both in United States and non-American clinical trial sites, and the individual trials were published in prestigious biomedical journals.

Additionally, Dr. Dere was director of the Lilly European Regulatory and Safety department based in UK from 1991-1994, and a member of the CIOMS 3 Pharmacovigilance Working Group, sponsored by the World Health Organization. He has participated in a number of regulatory hearings at both the FDA and European Medicine Agency for approvals of oral antibiotics; raloxifene and PTH 1-34 in osteoporosis; and panitumumab for colorectal cancer. He has actively participated in a number of publications authored by academic, industry, and regulatory personnel which have served as the basis for subsequent guidelines for the European Medicines Agency.

Malin Parmar, PhD

Malin Parmar is a professor in cellular neuroscience at Lund University in Sweden and a New York Stem Cell Foundation – Robertson investigator. She earned her Bachelor of Science at Simon Fraser University in Canada and her PhD in Medical Sciences and Developmental Biology at Lund University. Dr. Parmar completed two postdoctoral fellowships; one at Edinburgh University (financed by the Swedish Research Council) and another at Lund University.

Dr. Parmar's research has a strong translational focus. Together with her lab she has shown in a series of high-profile publications how human fibroblasts can be converted into neurons, how glial cells can be reprogrammed into neurons *in vivo*, and how therapeutic dopamine neurons can be generated from human embryonic stem cells. She is the recipient of an ERC starting grant and an ERC Consolidator grant. She leads the European effort STEM-PD, designed to bring stem cell-derived dopamine neurons to clinical trials, and she is a key partner within European and International networks as well as Industry partners to develop new, cell based therapies for Brain Repair with focus on Parkinson's Disease.

Proposed Third Term Reappointments to GWG

Last	First	Term	Expertise
Balber	Andrew	6	Immunology; Drug Development of Cellular Therapeutics
Cox	Charles	6	Pediatric Trauma; Neurologic Injury; Combination Products; Cardiopulmonary
DiPersio	John	6	Leukemias; Bone Marrow Transplantation
Furth	Mark	6	Fetal & Adult Stem Cells in Regenerative Medicine; Tissue Engineering; Muscular Myopathy
Gunter	Kurt	6	Regulatory Affairs & Therapy Development
Harfe	Brian	6	Developmental Biology; microRNA
Heimfeld	Shelly	6	Cellular Therapy; Hematology; GMP Cell Production
Rojas	Mauricio	6	Stem Cells in Lung Repair & Injury
Zwaka	Thomas	6	Pluripotent Stem Cell Biology; Molecular Genetics

Andrew E. Balber, PhD

Andrew Balber provides consulting services to corporate and academic clients developing cell therapy products. Dr. Balber also serves as a member of Grants Working Group of the California Institute of Regenerative Medicine. In this capacity, he reviews grant applications concerned with translational and clinical aspects of stem cell technologies. Previously, Dr. Balber was a Founder, and for ten years served as the Chief Scientific Officer, of Aldagen, Inc., a company developing cell products for use in transplantation and in therapy of cardiovascular diseases. He began his work in cell therapies in 1983 when, as a Duke University faculty member in immunology and Director of the Comprehensive Cancer Center Flow Cytometry Facility, he helped organize and manage a university-based, industry-sponsored research consortium in immunology. This organization developed technologies for the isolation, propagation, and commercial use of T- and B-lymphocyte populations and monoclonal antibodies. Subsequently, as the Associate Director of the Office of Science and Technology at Duke, Dr. Balber participated in building relationships with industrial partners in projects ranging from early research through health care delivery. He helped start companies based on Duke technology and was the liaison to the management of virtual companies working with university scientists. He played an important role in the commercialization of the technology that gave rise to Myozyme®, an approved therapy for Pompe Disease. In the area of cell therapies, Dr. Balber was centrally involved in two early important transactions – spinning out Merix Bioscience [subsequently Argos Therapeutics] and establishing a manufacturing facility initially used collaboratively by Applied Immune Sciences and Duke to produce cellular therapeutics. When he helped found and joined Aldagen in 2000, Dr. Balber had the opportunity to participate more directly in the development of stem cell therapies. He and his colleagues at Aldagen developed and launched Aldefluor®, a research use only product for identifying and isolating normal and cancer stem cells, and obtained FDA clearance to market a second product, Aldecount®, for clinical enumeration of these cells. Most significantly, he helped the Company establish and maintain a clinical program during which patients were treated under seven cleared INDs with products composed of ALDHbr cells for indications related to cord blood transplantation, critical limb ischemia, ischemic heart failure, and stroke. When Aldagen was acquired in 2010, Dr. Balber founded Cicada. He also served a Senior Scientific Advisor to the Robertson Clinical & Translational Cell Therapy Program at Duke University from 2012-2016. At Duke, he participated in submission of additional INDs for Phase 1 and

Phase 2 clinical trials involving use of umbilical cord blood cells and cell products manufactured from them to treat injuries and inherited rare metabolic diseases of the central nervous system. He directed IND-enabling research on characteristics and mechanism of action of these cell products.

Dr. Balber earned a BA from Haverford College (1966), completed a Ph.D. from Rockefeller University (1971), did post-doctoral work at Yale University (1971-1973), and taught undergraduates at Bates College (1973-1980). He operated an NIH-funded research program in parasite immunology and cell biology for 15 years at Duke before transitioning to a career in technology transfer and, then, to product development.

Charles S. Cox, Jr. MD

Chuck Cox is Professor of Pediatric Surgery, and the George and Cynthia Mitchell Distinguished Chair in Neuroscience, directing the Pediatric Surgical Translational Laboratories and Pediatric Program in Regenerative Medicine at the University of Texas Medical School at Houston. He is co-director of the Texas Trauma Institute and directs the Pediatric Trauma Program at the University of Texas-Houston/Children's Memorial Hermann Hospital in the Texas Medical Center.

A Texas native, Dr. Cox received his undergraduate degree from the University of Texas at Austin in the Plan II Liberal Arts Honors Program. Upon graduating from the University of Texas Medical Branch, he completed his Surgery residency at the University of Texas Medical School at Houston. Further post-graduate fellowships were completed in Pediatric Surgery at the University of Michigan, a NIH T32 sponsored clinical and research fellowship in cardiopulmonary support/circulatory support devices/bio-hybrid organs at the Shriner's Burns Institute, and Surgical Critical Care/Trauma at the University of Texas Medical School at Houston. He is certified by the American Board of Surgery in Surgery, with added qualifications in Pediatric Surgery and Surgical Critical Care. He served in Afghanistan with the 82nd Airborne in the 909th Forward Surgical Team in 2002.

The Pediatric Translational Laboratories and Pediatric Program in Regenerative Medicine represent a multi-disciplinary effort that addresses problems that originate with traumatic injury and the consequences of resuscitation and critical care. The Program focuses on progenitor cell based therapy (stem cells) for traumatic brain injury, and related neurological injuries (hypoxic-ischemic encephalopathy, stroke, spinal cord injury), recently completing the first acute, autologous cell therapy treatment Phase I study for traumatic brain injury in children, as well as a DOD funded Phase 1/2a trial for severe TBI in adults (2015). Recently, the NIH funded Phase IIb clinical trial for cellular therapies in children with severe TBI completed with positive results; and the DOD Joint Warfighter Program has funded a Phase 2b trial in adults (2016-2022). The program has been continuously funded since 1998 through the National Institutes of Health, Department of Defense/MRMC, Texas Higher Education Coordinating Board/Emerging Technology Funds, Industry Collaboration (Athersys, Inc.; Celgene Cellular Therapeutics; CBR, Inc; HopeBio, Inc., Biostage, Inc.), and philanthropic contributions. The Program is housed in state-of-the-art laboratory facilities (4500 sf), and includes two cGMP, Class 10,000 facilities for the production of clinical grade cell and tissue products: Hoffberger Cellular Therapeutics Laboratory and the Griffin Stem Cell Therapeutics Research Laboratory. Other major areas of interest include: (1) resuscitation induced organ edema and dysfunction, (2) the neuroinflammatory reflex, (3) mesenchymal stromal cell exosomes as anti-inflammatory agents, and (4) mechanotransduction of stem cells to enhance their anti-inflammatory properties. Three biotechnology start-ups have arisen out of this work: EMIT Corporation, Coagulex, Inc., and Cellvation, Inc., and funded via various venture groups. He serves on Scientific Advisory Board positions with Biostage and CBR, Inc. He serves as a permanent member of the NIH/NINDS BINP study section and on the CIRM Grants Working Group review panel.

He is the author of over 250 scientific publications, 20 book chapters, and is the editor of texts entitled, *Progenitor Cell Therapy for Neurological Injury and Cellular Therapy for Neurological Injury*.

John DiPersio, MD, PHD

John DiPersio is Chief of the Division of Oncology, Deputy Director of the Siteman Cancer Center at Washington University School of Medicine in St. Louis, and the Virginia E. and Samuel J. Golman Professor of Medicine. Dr. DiPersio is an internationally recognized expert in hematopoietic stem cell transplantation and acute leukemia. His research focuses on fundamental and translational aspects of leukemia and stem cell biology.

Over the past 30 years, Dr. DiPersio has established himself as a leader in the field through his leadership and membership on committees and organizations such as American Society of Hematology (ASH), multiple NIH, CIRM, LLS and CPRIT Study Sections, NIH Special Emphasis Panels and the NCI's Board of Scientific Counselors. His >420 papers and consistent peer-reviewed funding demonstrate his scientific expertise. He is an elected member of ASCI and AAP, past president of the ASTCT (2018-19) and the recipient of the prestigious AACR Joseph H. Burchenal Memorial Award for Outstanding Achievement in Clinical Cancer Research in 2014, the ASH Mentor Award for Clinical Investigation in 2014 and a NCI R35 Outstanding Investigator Award in 2017.

Dr. DiPersio played a key role in the clinical development of plerixafor as a mobilizing agent for stem cell transplantation. His group was the first to show that disruption of the interaction of AML with bone marrow stromal cells using CXCR4 inhibitors sensitized AML cells to chemotherapy. His recent studies have focused on the development of novel methods of targeting the hematopoietic niche through the development of highly active small molecule inhibitors of CXCR4, VLA-4 and agonists of CXCR2 for both stem cell mobilization and chemosensitization.

Dr. DiPersio was the first to implicate JAK1/2 signaling in GvHD via genetic and pharmacologic inhibition of INF□R and IL-6R pathways. His preclinical work and early clinical trials led to the first drug ever approved by the FDA (ruxolitinib) for the treatment of steroid refractory acute GvHD. Recent studies by the DiPersio lab have uncovered the mechanisms by which JAK inhibitors alter T cell biology and have led to the identification of 'best-in-class" JAK inhibitors for the prevention and treatment GvHD in man.

Dr. DiPersio played a critical supporting role in the sequencing of the first cancer genome and a leadership role in identifying genetic and epigenetic factors that contribute to relapse in AML. He was the first to show that rare subclones present at the time of diagnosis often are responsible for AML relapse3. Recently, his group was also the first to show that epigenetic downregulation of HLA Class II antigens on AML blasts was associated with immune escape and relapse after allogeneic transplantation4. These observations have profound implications for how we diagnose, stratify and treat patients with AML.

Mark Furth, PhD

Mark Furth is Scientific Director for the Fibrolamellar Cancer Foundation, a nonprofit organization dedicated to finding new therapies for fibrolamellar carcinoma, a rare liver cancer affecting predominantly adolescents and young adults. Previously, he was Executive Director at Wake Forest Innovations, the commercialization enterprise of Wake Forest Baptist Medical Center. In prior positions at Wake Forest he led translational programs in the Institute for Regenerative Medicine and the Comprehensive Cancer Center. Furth worked for 18 years as an executive in the biopharmaceutical industry, leading research programs in regenerative medicine, cancer, and drug discovery. He was the first scientist at Regeneron, where his team identified several neurotrophic factors that entered clinical testing. As VP for Molecular Sciences at the Glaxo Research Institute (now GlaxoSmithKline) he helped to introduce genomics into drug discovery and supervised biochemistry and structure-based drug design technologies. As Chief Executive Officer of Ingenex Inc., a subsidiary of Titan Pharmaceuticals, Furth oversaw an early clinical trial of gene-modified hematopoietic stem cells in cancer patients, and the use of functional genomics to identify drug targets. When Pharmaceutical Product Development acquired Ingenex's functional

genomics technology and a combinatorial chemistry unit, Furth became Chief Scientific Officer of PPD Discovery. He subsequently served as head of research for early stage biotechnology companies in pharmacogenomics (PPGx, a joint venture of PPD and Sequana Therapeutics) and regenerative medicine (Incara, Endogeny Bio). In collaboration with his wife, Lola Reid (University of North Carolina at Chapel Hill), Furth has contributed to the identification of human liver and biliary tree stem cells that are candidates to treat hepatic disorders and diabetes.

Kurt Gunter, MD

Kurt Gunter is the Chief Medical Officer at Kuur Therapeutics. Dr. Gunter has devoted his career to the development of cell and gene therapies and his work experience has included the FDA as a Medical Officer in the Center for Biologics, Deputy Director of the FDA Division of Cell and Gene Therapy, Assistant Professor at Children's National Medical Center in Washington DC and several leadership positions in private industry (including Transkaryotic, ViaCell and Hospira). Kurt earned his MD from the University of Kansas and also has a BS in biological sciences, with distinction, from Stanford University. His postdoctoral training included Johns Hopkins and the US National Institutes of Health.

As past President of the International Society for Cellular Therapy (ISCT), Kurt played a worldwide leadership role in promoting understanding of the clinical, regulatory, manufacturing, and marketing requirements for the successful development of cell and gene therapies. He is an active member of several other scientific societies including American Association of Clinical Pathologists (Fellow), American Association of Blood Banks, American Society for Clinical Oncology, American Association of Immunology, International Society for Stem Cell Research, American Society of Hematology, and the American Society for Transplantation and Cellular Therapy.

Brain Harfe, PhD

Brian Harfe is Associate Dean for Research and Associate Dean for the Natural Sciences and Mathematics in the College of Liberal Arts and Sciences (CLAS). In addition, he is Senior Assistant Provost for the Office of Teaching and Technology. As Associate Dean, he is responsible for the 7 science/math departments and oversees >$100 million dollars. In addition, he develops new curriculum and leads all online initiatives in CLAS. As Senior Assistant Provost he is responsible for implementing new initiatives that enhance the UF learning environment and increase access to UF. He is the course director for the Genetics and Health course (designed course) for first year medical students and designed an innovated online undergraduate upper division biology course that includes 100% online laboratories. During the COVID19 pandemic, he played a key role in transitioning the University to a primarily online course modality and ensuring the safe continuation of research activities on the UF campus.

Dr. Brian Harfe earned a BS (honours) degree from the University of Glasgow, Scotland, followed by a Ph.D. in developmental biology from The Johns Hopkins University. After completing his Ph.D., he held postdoctoral positions at Emory University and Harvard Medical School. Dr. Harfe's area of research uses the mouse and chick model systems to investigate how limbs and the intervertebral disks form. He has published >100 peer-reviewed papers, which have been cited >16,900 times.

Shelly Heimfeld, Ph.D.

Shelly Heimfeld is recently retired. Previously he was a founder and Executive Vice President responsible for Manufacturing and Research at Nohla Therapeutics, a Full Faculty Member at the Fred Hutchinson Cancer Research Center, Scientific Director for the Hutch's cGMP Cell Processing Facility at the Fred Hutch where all extensively manipulated experimental cell therapies were produced, and the Laboratory Director for the Cellular Therapy Laboratory at the Seattle Cancer Care Alliance where all minimally manipulated cell components for treatment of patients at the Center are processed. His primary

responsibilities were to ensure safety, quality, and effectiveness of each cell product, along with implementation of new technologies, translation of basic procedures into compliant clinical protocols, product development, process improvement, and regulatory compliance.

Dr. Heimfeld received his Ph.D. from UC Irvine, completed postdoctoral studies with Dr. Irv Weissman at Stanford before going into industry as a founding scientist at SyStemix and later at CellPro, Inc, the first company to develop an FDA approved device for $CD34^+$ cell enrichment. Dr. Heimfeld is a Past-President of ISCT and continues to work with granting, regulatory, and other organizations to facilitate exchange of ideas and best practices in the rapidly evolving area of Cell Therapy.

Mauricio Rojas, MD

Mauricio Rojas is an Associate Professor and Scientific Director of the Simmons Center for Interstitial Lung Diseases at the University of Pittsburgh. Dr. Rojas received his undergraduate medical degree from the National University of Colombia and completed his postdoctoral training in Immunology at the Institute of Immunology, Colombia and Vanderbilt University. He has been a member of the American Thoracic Society since 2002. Dr. Rojas is a co-founder of the Stem Cell Working Group (2009) and a founder of the Aging Working Group, which he currently chairs. He received the Service Award from the RCMB Assembly in 2011 and the Carol Basaum Award in 2014.

Dr. Rojas is a pioneer in the research of Mesenchymal Stem Cells as a mechanism of lung repair and the study of lung aging as a critical factor in the vulnerability to lung diseases. His ongoing projects include functional consequences of age-related exhaustion and senescence of mesenchymal cells and the development of human preclinical models for lung therapies. Emory University recognized his contributions with the Pulmonary Star Award in 2007 and the Early Career Award from the Department of Medicine in 2009. Subsequently, Dr. Rojas received the Dorothy Dillon Ewason Lecture Award for the American Federation of Aging (AFAR) in 2009, the Established Investigator Award from the Pulmonary Fibrosis Foundation in 2013, and the Faculty Translation Award from the Division of Pulmonary at the University of Pittsburgh. Dr. Rojas's research focuses on aging of stem cells and disease susceptibility and cell therapy for the treatment of acute and chronic respiratory distress syndrome

Dr. Rojas is an Editorial Board Member of several publications, namely *OA Nutrition & Dietetics, Journal of Allergy & Therapy, Stem Cell Dependent Therapies in Chronic Inflammatory Disorders, JSM Regenerative Medicine,* and *Chronicles of Surgery*.

Thomas Zwaka, MD, PhD

Thomas Zwaka is Director of the Icahn School of Medicine at Mount Sinai Black Family Stem Cell Institute and editor-in-chief of Stem Cell Research.

Dr. Zwaka was recruited to Mount Sinai in 2013 to become Professor of Developmental and Regenerative Biology. Within a year, he established the Huffington Foundation Center for Cell-Based Research in Parkinson's Disease, which he also directs, as a collaborative effort to develop better treatments for this all-too-common neurodegenerative disease.

After earning his MD and Ph.D. degrees from Ulm University in Germany, Dr. Zwaka trained as a cardiologist and discovered the link between C-reactive protein and atherosclerotic inflammation, a connection that has had enormous importance for cardiology. Dr. Zwaka then went to the University of Wisconsin to do his postdoctoral fellowship in the lab of Jamie Thomson, who derived the first human embryonic stem cell line in 1998.

In Thomson's lab, Dr. Zwaka pioneered methods to genetically manipulate stem cells, resulting in studies noted in publications that have been cited more than a thousand times. He then joined the faculty of Baylor College of Medicine, serving in both the Department of Molecular and Cellular Biology and in the Center for Cell and Gene Therapy. Within a few years, he became Co-Director of the Stem Cells and Regenerative Medicine Center. At Baylor, the Zwaka Lab discovered a key regulator of pluripotency that behaved so differently from canonical stem cell factors that it was named Ronin.