CIRM
CALIFORNIA'S STEM CELL AGENCY

Nominations for Appointment to the Grants Working Group (GWG)

Appointment of New Members

Frank W. Rockhold, PhD
Frank has had diverse leadership and consulting experience in industry and academia including clinical trials design, data monitoring, data standards, benefit/risk, epidemiology, real world evidence, cardiovascular research, and most recently safety and pharmacovigilance. Now a full time Professor of Biostatistics and Bioinformatics at Duke Clinical Research Institute, Frank's 40 year career includes senior research positions at Lilly Research Laboratories, Merck Research Laboratories and GlaxoSmithKline, where he recently retired as Senior Vice President of Global Clinical Safety and Pharmacovigilance. He has been a leader in the scientific community in promoting data disclosure and transparency in clinical research. Frank served for 9 years on the board of directors of the non-profit CDISC, most recently as Chairman and is past president of the Society for Clinical Trials. He is a member of the PCORI Advisory Panel on Clinical Trials and on the board of the Frontier Science and Technology Research Foundation. Frank has more than 75 publications in major scientific journals across a wide variety of topics and has held faculty appointments at five other universities, including a current post as Affiliate Professor of Biostatistics at Virginia Commonwealth University Medical Center. He is also currently Managing Partner of HunterRockhold, Inc., which provides strategic consulting to Industry, Government, and Academia in the areas of clinical trials, safety and pharmacovigilance, data disclosure and transparency. He holds a BA in Statistics from The University of Connecticut, an ScM in Biostatistics from The Johns Hopkins University, and a PhD in Biostatistics from the Medical College of Virginia. Frank is a Fellow of both the American Statistical Association and the Society for Clinical Trials and an Accredited Professional Statistician, PStat®.

Reappointment of Scientific Members to the Grants Working Group

Grants Working Group Members originally appointed in 2008-10 have terms that are now expiring or just expired. We are seeking the reappointment of the individuals listed in the table below. Their updated biographies follow. In accordance with the rules set forth by Proposition 71, reappointments should be staggered into thirds, each with a 2, 4, or 6-year term.

Proposed Reappointments to GWG

Last	First	Term	Expertise
Brundin	Patrik	6	Neural Transplantation (PD)
Diamond	Marc	2	Medicine Research & Development
Hassell	John	4	Cancer Biology (Breast); Therapeutics; Cell Signaling; Functional Genomics
Hefti	Franz	6	Drug Development in Neuroscience (AD); Neuroscience Biology
McKenna	David	2	Development of Cellular-based Therapies; GMP Manufacturing of Adult Stem Cells
Palecek	Sean	6	Pluripotency; Tissue Engineering; Biosensors
Ross	Theodora	4	Hematopoiesis; Leukemia
Wirostko	Barbara	4	Ophthalmology; Clinical Development

Patrik Brundin
Patrik Brundin is the Associate Director of Research at Van Andel Research Institute, Director of Van Andel Research Institute's Center for Neurodegenerative Science, and the Jay Van Andel Endowed Chair in Parkinson's Research. He obtained his M.D. (1992) and Ph.D. (1988) at Lund University in Sweden where he was a professor of neuroscience in the Department of Experimental Medical Science Neuronal Survival Unit in the Wallenberg Neuroscience Center from 2000–2014.

Dr. Brundin is a leader in the field of Parkinson's disease and was a part of the research team that first developed a clinical procedure for intracerebral transplantation in this disease. He leads a research group that is active in several areas of investigation related to experimental models of neurodegenerative diseases. His main current research interests are the alpha-synuclein prion-like hypothesis, drug repurposing and development of disease-modifying therapies in Parkinson's disease.

Dr. Brundin has published over three hundred papers on Parkinson's disease and closely related topics. He is co-editor-in-chief of the *Journal of Parkinson's Disease*. In 2001, he was identified as one of the 0.5% most cited scientists in his area, and he has received numerous awards for his work including his election as a member of Academia Europaea. He has edited a book on restorative therapies in Parkinson's disease, is a member of the editorial board of several renowned journals, and is a consultant for biotechnological and pharmaceutical companies. He as served as a grant reviewer for agencies including The Wellcome Trust, UK and the Medical Research Council (MRC). From 1995-2000 he was president of the Network for European CNS Transplantation and Repair (NECTAR).

Marc Diamond, MD
Marc Diamond is the founding Director of the Center for Alzheimer's and Neurodegenerative Diseases, and is a Professor of Neurology and Neurotherapeutics. Dr. Diamond completed an internship, residency, and chief residency in neurology at the University of California, San Francisco (UCSF) in 1997. After a postdoctoral fellowship, he was a faculty member in the Neurology Department at UCSF from 2002-2009. From 2009-2014 he was the David Clayson Professor of Neurology at Washington University in St. Louis, before he was recruited to UT Southwestern.

His research focuses on molecular mechanisms of neurodegeneration in Alzheimer's disease and related disorders, with the goal of developing novel therapies and diagnostic tools. A therapeutic antibody he co-developed at Washington University in St. Louis is now entering clinical trials for treatment of dementia. The Center for Alzheimer's and Neurodegenerative Diseases is comprised of a multidisciplinary group of investigators who are focused on understanding the basis of progressive protein aggregation in human disease. They are using this knowledge to hasten the day when neurodegeneration can be detected presymptomatically and stopped before it causes disability.

Dr. Diamond has received numerous awards, has published in peer reviewed journals, and has been issued 2 patents. Dr. Diamonds has served as a referee for numerous journals including the Proceedings of the National Academy of Sciences, Nature Chemical Biology, Journal of Cell Biology, and Journal of Neuroscience and has participated in peer review of grant applications including serving as a study section member at the National Institutes of Health (NIH).

John A. Hassell, PhD
Dr. John A. Hassell is a Professor in the Department of Biochemistry and Biomedical Sciences, and Director of the Centre for Functional Genomics at McMaster University in Hamilton, Ontario, Canada. Dr. Hassell received his Ph.D. in Molecular Biology from the University of Connecticut; his Ph.D. thesis research was centered on understanding the differences in gene expression between normal cells and their cancer cell counterparts. He completed his postdoctoral studies at the Cold Spring Harbor Laboratory with Dr. Joe Sambrook. Dr. Hassell was one of several postdoctoral fellows who co-discovered RNA splicing, a discovery that led to a Nobel prize to Dr. Richard Roberts of the Cold Spring Harbor Laboratory. Dr Hassell moved to Canada in late 1977 and spent 9 years as an Assistant and Associate Professor at McGill University before moving to McMaster University in 1988 as the first Director of the Institute for Molecular Biology and Biotechnology, a position he held for 12 years.

Dr. Hassell's current research programs are centered on the role of transcription factors in breast cancer, breast cancer stem cells, and mammary gland development. His laboratory discovered a key new transcription factor, Pea3, and showed that this transcription factor is overexpressed in human and mouse breast tumors and is required for the

progression of breast cancer. More recently his laboratory has identified breast cancer stem cells in mouse breast tumors and is identifying biomarkers and molecular therapeutic targets in these cells. In addition, Dr. Hassell has defined condition to propagate mammary epithelial stem/progenitor cells and breast cancer stem cells in vitro under conditions that preserves their phenotype. The availability of these cell cultures has afforded him the opportunity to perform high-throughput phenotypic screens to identify compounds that selectively kill breast cancer stem cells. A screen of 30,000 compounds led to the discovery of cancer-cell specific lead compounds are currently being developed in collaboration with medicinal chemists at McMaster University.

Dr. Hassell has over 70 publications in high-profile journals and maintains a well-funded research program. He has served as a member and chair of numerous grant review committees and study sections of the Canadian Institutes for Health Research, National Institutes of Health, and the National Cancer Institute of Canada. He also has served on various advisory boards and is currently a member of the Research Advisory Committee of the Canadian Breast Cancer Research Alliance. Dr. Hassell is also a key member of the Stem Cell Network, a Canadian National Centre of Excellence, and is the Project Director of a collaborative research program to identify new therapeutic agents that target cancer stem cells, which involves Canada's leading cancer stem cell investigators. Dr. Hassell currently is spearheading the search for funding a Cancer Stem Cell Consortium, a collaborative group of internationally renowned cancer stem cell researchers in Canada and California, under the auspices of the Canada-California Strategic Innovation Partnership.

Franz F. Hefti, PhD
Franz Hefti is President and Chief Operations Officer at NeuroPhage Pharmaceuticals, a biotech company developing drugs targeting aggregates of misfiled proteins. In addition to his role at NeuroPhage Pharmaceuticals, Dr. Hefti serves as advisor to several biotech companies developing therapeutics for neurological diseases. Prior to joining NeuroPhage, he was chief scientific officer at Avid Radiopharmaceuticals Inc., a company that developed imaging diagnostics and which was acquired in 2010 by Lilly. He also was executive vice president of drug development at Rinat Neuroscience, a company with antibody programs for pain and neurological indications that was acquired by Pfizer in 2006. He has been co-founder, board member, or scientific advisor to several other start-up biotech companies in the United States and Europe.

Before engaging in biotech companies, Dr. Hefti held senior management positions at Merck & Co. and Genentech. Prior to Genentech, he spent more than a decade in academia as a Professor at the University of Southern California and Associate Professor at the University of Miami, where he carried out seminal research on therapeutic applications of neurotrophic factors.

Dr. Hefti has published over 250 papers on neurotrophic factors and topics in neuropharmacology, as well as the textbook *Drug Discovery for Nervous System Diseases.* He received his PhD from the University of Zurich and completed postdoctoral research at the Massachusetts Institute of Technology and the Max Planck Institute in Munich.

David McKenna, Jr, MD
Dr. McKenna is the Director of the Division of Transfusion Medicine of the University of Minnesota Medical School. He is also the Scientific and Medical Director of Molecular & Cellular Therapeutics, the University's cGMP facility, as well as Laboratory and Medical Director of the University of Minnesota Medical Center Clinical Cell Therapy Laboratory and the director of the fellowship program in Transfusion Medicine/Blood Banking. He is an Associate Professor in the Univesity of Minnestoa Medical School. Dr. McKenna received his B.S. degree from the University of Notre Dame and his M.D. from the Saint Louis University School of Medicine. He completed a residency in Anatomic and Clinical Pathology with an emphasis on cell therapy at the University of Minnesota and the University of Vermont and completed a fellowship in Transfusion Medicine at the University of Minnesota. Dr. McKenna is a board certified physician in Anatomic and Clinical Pathology and Blood Bank/Transfusion Medicine licensed to practice medicine in the State of Minnestoa.

Dr. McKenna's research centers on the translational ("bench to bedside") development of cell based therapies. Current and developing projects include clinical trials with natural killer cells [peripheral blood derived, umbilical cord blood (UCB) derived] and T regulatory (CD4+/CD25+) cells (peripheral blood derived, umbilical cord blood derived) in support of bone marrow transplantation, allogeneic large multivalent immunogen (LMI) vaccine [cell line based cancer vaccine: melanoma (SK23) and breast cancer (SKBR3)], marrow mononuclear cells and cardiosphere derived cells for cardiac regenerative medicine, skeletal myoblasts, dendritic cell based vaccines, and mesenchymal stem/stromal cells (MSCs) for a variety of

applications. His pre clinical research focuses on the isolation, characterization, expansion, and differentiation of stem cells from human UCB.

Dr. McKenna was key investigator in the National Heart, Lung, & Blood Institute (NHLBI) sponsored PACT (Production Asisstance for Cellular Therpay) group. He is a member of the International Society for Stem Cell Research, the Tissue Engineering/Regenerative Medicine International Society, and the American Society for Hematology and the American Association of Blood Banks (AABB), serving on the board of directors for four years and leading the AABB Cellular Therapy Section Coordinating Committee for several years. He is also a Scientific Member of the Biomedical Excellence for Safer Transfusion (BEST) Collaborative Cellular Therapy Team. Dr. McKenna has published extensively and has participated as a peer reviewer for several granting agencies and journals.

Sean Palecek, PhD
Sean Palecek is the Milton J. and Maude Shoemaker Professor and a Vilas Distinguished Achievement Professor in the Department of Chemical & Biological Engineering at the University of Wisconsin – Madison. Sean received a bachelor's degree in chemical engineering at the University of Delaware. He performed his graduate research with Doug Lauffenburger and Rick Horwitz, earning an M.S. in chemical engineering from the University of Illinois at Urbana-Champaign and a Ph.D. in chemical engineering from MIT. Sean performed postdoctoral research in molecular genetics and cell biology at the University of Chicago under the supervision of Steve Kron.

Sean's lab studies how human pluripotent stem cells (hPSCs) sense and respond to microenvironmental cues in making fate choices, with a focus on differentiation to cardiovascular lineages. They construct culture systems that apply soluble chemical signaling, immobilized matrix proteins, cell-cell contacts, and mechanical cues to differentiating hPSCs and determine the developmental pathways stimulated by these cues. Then they use this information to engineer simple, efficient, and robust process to differentiate hPSCs to desired somatic cell types. Sean's engages in highly collaborative, team-based research working with labs in biology, medicine, physical sciences, and engineering to tackle problems at the forefront of regenerative medicine. Sean's lab has generated novel mechanistic insight and developed protocols for differentiation of hPSCs to cardiomyocytes, vascular endothelial cells, brain microvascular endothelial cells, epidermal keratinocytes, vocal fold epithelial cells, and simple epithelial progenitors. They strive to engineer fully-defined, animal component-free differentiation platforms, compatible with biomanufacturing of cells for *in vitro* and *in vivo* applications. These differentiation systems have been published in top scientific journals including the *Proceedings of the National Academy of Sciences*, *Nature Biotechnology*, and *Nature Methods*. Sean's lab has multiple patents on stem cell-derived cell types, and several of these patents are licensed by pharmaceutical and tissue engineering companies.

Sean's recent awards include the Cozzarelli Prize of the National Academy of Sciences and the Biotechnology Progress Excellence in Research Publication Award for his work on cardiovascular tissue engineering from hPSCs. He is a leader in the field of Stem Cell Engineering. He chaired the 3rd International Conference on Stem Cell Engineering, co-sponsored by ISSCR and SBE, in 2012 and is on the executive committee for BMES, SBE, TERMIS, ISSCR, and ACS meetings. He has served on numerous federal and state stem cell advisory boards. He is on the executive committee of a NIST Cell Manufacturing Consortium Planning Project and was a member of an NSF/NIH/NIST International Assessment of Stem Cell Engineering. Sean has experience in leadership positions, serving as the Bioengineering Thrust Leader for the UW Stem Cell and Regenerative Medicine Center and the cell culture co-director for the UW Skin Disease Research Center.

Theodora Ross, MD, PhD
Theodora Ross holds the Jeanne Ann Plitt Professorship in Breast Cancer Research and the H. Ben and Isabelle T. Decherd Chair in Internal Medicine, in Honor of Henry M. Winans, Sr., M.D. at UT Southwestern Medical Center. She specializes in oncology and cancer genetics.

Dr. Ross received her M.D. and Ph.D. from the Washington University Medical Scientist Training Program (MSTP) in St. Louis. She completed her medical residency in Boston at Harvard's the Brigham and Women's Hospital, followed by a fellowship in oncology at the Dana-Farber Cancer Institute.

Prior to joining UT Southwestern, Dr. Ross served as a clinician and researcher at the University of Michigan in Ann Arbor, where for many years she cared for women with breast cancer and investigated the basic cellular mechanisms of

cancer cells and how those cells resist targeted cancer drugs. She also served as the associated director of the University's MSTP.

Her laboratory at UT Southwestern continues this research and also investigates BRCA1, a breast and ovarian cancer susceptibility gene. She has received numerous cancer research related honors including awards from the American Cancer Society, the American Society of Hematology, the Damon Runyon Cancer Research foundation, the Burroughs Wellcome Fund, and the Leukemia and Lymphoma Society.

In her clinical practice, Dr. Ross cares for individuals at a high genetic risk for any type of cancer. She also serves as the director of the UT Southwestern Cancer Genetics Program.

Outside of her official job, Dr. Ross has been a strong advocate for funding of basic cancer research and has used her experience with the Washington University and University of Michigan MST Programs to help recruit for and grow UT Southwestern's MSTP, which trains the next generation of physician scientists.

Dr. Ross has served as the Secretary/Treasurer for the American Society for Clinical Investigation and currently serves on the Editorial Board of *Clinical Genetics* and the Board of Consulting Editors for the *Journal of Clinical Investigation.*

Barbara Wirostko, MD
Barbara Wirostko, a board certified Ophthalmologist and fellowship trained glaucoma specialist, is co-Founder and Chief Scientific Officer of Jade Therapeutics, based in Salt Lake City, developing a novel drug delivery hyaluronic acid polymer platform to treat ophthalmic diseases of unmet need. Jade is now a wholly owned subsidiary under EyeGate Pharmaceuticals, and Dr. Wirostko has assumed the Chief Medical Officer for EyeGate. Jade had been recognized by the state of Utah as an exciting emerging Biotech Company. Dr. Wirostko as Jade's PI, has been awarded upwards of 3 million in SBIR Phase 2 federal funding from the National Science Foundation and the Dept. of Defense. Prior to Jade she was Chief Medical Officer of Altheos, a biotech company developing a ROCK inhibitor for glaucoma. She maintains an academic research and clinical practice at the Moran Eye Center, University of Utah and holds both a Clinical Adjunct Associate Professor in Ophthalmology and as Adjunct Associate Professor of Bioengineering position within the University.

Barbara previously worked as Senior Medical Director and the Glaucoma Medical Team Lead within the Specialty Care Business Unit, Clinical Development and Medical Affairs division at Pfizer in NY. She oversaw the development of pipeline glaucoma strategy as well as the Medical Affair programs for the global product Xalabrands. She had direct involvement with development programs, clinical trials and post marketing medical responsibilities within Ophthalmology. She was directly responsible and involved with a successful Phase 3 pediatric regulatory filing to the European regulatory agency. The Xalatan pediatric development team under her lead was awarded the 2010 Pfizer Team Award for outstanding performance.

Prior to joining Pfizer, Barbara was Chief Ophthalmologist practicing as a clinician and specializing in glaucoma at the Huntington Medical Group PC, in Huntington, NY. During her 10 years in practice, she served as Principle Investigator for various pivotal glaucoma clinical trials for major Pharmaceutical companies specialized in eye care. She has written numerous peer reviewed articles, has served on journal editorial review boards, advisory boards for drug development and has lectured worldwide in the area of Ophthalmology. She is an active member and fellow of the American Academy of Ophthalmology, and a member of Association for Research in Vision & Ophthalmology, American Glaucoma Society and local state societies. She was awarded a Distinguished Alumni Award from Columbia University Edward S Harkness Eye Institute.