

**CIRM Scientific and Medical Research Funding Working Group
Biographical information of candidates nominated to serve as
Scientific Members of the Working Group**

Bradley E. Bernstein, MD, PhD

Dr. Bernstein is Associate Pathologist at Massachusetts General Hospital, Associate Professor of Pathology at Harvard Medical School, and Senior Associate Member at the Broad Institute. He is also an Early Career Scientist of the Howard Hughes Medical Institute. He co-directs the Epigenomics Program at the Broad Institute, and serves as a principal investigator in the Broad's Klarman Cell Observatory and is affiliated with Massachusetts General Hospital's Center for Systems Biology and Center for Cancer Research and the Harvard Stem Cell Institute. Dr. Bernstein received his BS from Yale University in physics and his MD and PhD from the University of Washington School of Medicine. After receiving his MD and PhD, Dr. Bernstein completed a residency in clinical pathology at Brigham and Women's Hospital. He carried out postdoctoral research at Harvard University with Stuart Schreiber and also collaborated extensively with Eric Lander. He joined the faculty of Massachusetts General Hospital and Harvard Medical School in 2005.

Dr. Bernstein's research focuses on epigenetics - changes in gene activity governed by influences outside the genes themselves - and specifically how modifications to the protein scaffold called chromatin contribute to mammalian development and human cancer. His laboratory is characterizing epigenetic mechanisms that underlie stem cells' ability to give rise to almost any kind of cell, while also exploring how epigenetic mechanisms contribute to malignant transformation and therapeutic resistance.

Dr. Bernstein oversees two major NIH projects at the Broad Institute. These include the NHGRI-sponsored ENCODE project, which seeks to catalog all of the working parts of the genome, and the Epigenomics Project, which produces reference epigenomes for human tissues and stem cells. This work benefits from an outstanding team of production-oriented scientists in the Epigenomics Program and extensive collaborations with the sequencing center, computational scientists, and disease researchers at the Broad Institute.

Dr. Bernstein's honors and awards include a Howard Hughes Postdoctoral Research Fellowship for Physicians, a Young Investigator Award from the Academy of Clinical Laboratory Physicians, a Career Award in the Biomedical Sciences from the Burroughs Wellcome Fund, a junior faculty award from the Culpeper Foundation, a Howard Goodman fellowship, and the Martin Prize in Basic Research from Massachusetts General Hospital.

Richard A. Gibbs, PhD

Dr. Gibbs is the Wofford Cain Chair in Molecular and Human Genetics, Professor in the Department of Molecular and Human Genetics, and Director of the Human Genome Sequencing Center at Baylor College of Medicine. He earned his BSc (Hons)

and his PhD in Genetics and Radiation Biology at the University of Melbourne and completed a postdoctoral fellowship at Baylor College of Medicine studying the molecular basis of human X-linked diseases and development of technologies for rapid genetic analysis. In 1991, Dr. Gibbs joined the faculty at BCM and played a key role in the early planning and development phases of the Human Genome Project (HGP). In 1996, he established the Human Genome Sequencing Center (HGSC) that subsequently was chosen to be one of five worldwide sites to complete the final phase of the project. The HGSC contributed approximately ten percent of the HGP and it was completed in 2004.

The HGSC now occupies more than 36,000 square feet, employs over 200 staff including eighteen faculty. The group collaborated to sequence the first species of fruit fly, *Drosophila melanogaster*, the Brown Norway rat and rhesus macaque. The group independently completed the second species of fruit fly, *Drosophila pseudoobscura*, the honeybee, wasp, flour beetle, the bovine genome, the sea urchin, *Dictyostelium discoideum* and innumerable bacteria. The BCM-HGSC also engaged in a program to sequence all human cDNAs, create the human and bovine haplotype maps and more recently, the cancer genome project. In 2007, the group produced the first sequence of a diploid human, James Watson. In that year a new method for capture and analysis of human DNA was developed.

Current research within the HGSC is focused upon the genomics of cancer, heart disease and autism. To achieve this the group is sequencing single human genomes at an increasing rate. New molecular technologies are being developed for the mapping and sequencing, for exploring novel chemistries for DNA tagging, and to enable development of instrumentation for DNA manipulation. The HGSC is also part of the Human Microbiome Project and has an active bioinformatics program, with research projects involving biologists and computer scientists. Problems under study focus on developing tools for generating, manipulating, and analyzing genome data.

Dr. Gibbs has received many awards and honors including election to membership in the Institute of Medicine (IOM), receipt of LSU Chancellor's Distinguished Lectureship, and the Michael E. DeBakey, MD Excellence in Research Award.

Martin F. Pera, PhD

Dr. Pera is Professor of Stem Cell Sciences at the University of Melbourne, the Florey Neuroscience Institute, and the Walter and Eliza Hall Institute for Medical Research. He serves as Program Leader for Stem Cells Australia, the Australian Research Council Special Research Initiative in Stem Cell Sciences (www.stemcellsaustralia.edu.au). He received his BA degree in English Language and Literature from the College of William and Mary and his PhD in Pharmacology from George Washington University. Dr. Pera carried out postdoctoral research at the Institute of Cancer Research and the Imperial Cancer Research Fund in London, and was a Research Fellow at the Department of Zoology at Oxford University. Thereafter he moved to Australia where he became Research Professor at the

Monash Institute of Medical Research at Monash University and the Director of Embryonic Stem Cell Research at the Australian Stem Cell Centre. He served as Professor and Founding Director of the Eli and Edythe Broad Center for Regenerative Medicine and Stem Cell Research at the Keck School of Medicine of the University of Southern California from 2006 before returning to Melbourne in 2011.

Dr. Pera's research interests include the cell biology of human pluripotent stem cells (PSCs), early human development, and germ cell tumors. Dr. Pera was among a small number of researchers who pioneered the isolation and characterization of PSCs from human germ cell tumors of the testis, work that provided an important framework for the development of human embryonic stem cells (ESCs). His laboratory at Monash University was the second in the world to isolate ESCs from the human blastocyst, and the first to describe their differentiation into somatic cells in vitro. He has provided extensive advice to state, national and international regulatory authorities on the scientific background to human ESC research.

Dr. Pera's research group focuses on the extrinsic factors (signals from outside the cell) involved in maintenance of the pluripotent state in human ESCs, and those factors that drive stem cell commitment into progenitor cells representative of the three embryonic germ layers (the precursors of the primordia that form the tissues of the body). A major hypothesis behind this work is that PSC cultures represent an interactive hierarchy of different cell types, similar to those found in the embryo around the time of implantation into the womb, and that just as communication between different cell populations in the embryo acts to specify cell fate during development, similar conversations control stem cell maintenance and early lineage choice in vitro. This work has fundamental importance for our understanding of stem cell biology, but it also addresses practical questions that must be solved before human ESCs can achieve their full potential in research and medicine. Recently the Pera laboratory has focused on specification PSCs to the neural lineage and their differentiation into the cells that form the cerebral cortex. The cerebral cortex is that part of the brain that is uniquely human, and PSCs provide us for the first time with insights into how the cortex is formed, and into many important disorders, including epilepsy, schizophrenia and autism, that are thought to arise during this stage of brain development.

Dr. Pera serves on the Steering Group of the International Stem Cell Initiative, was on the advisory board of the National Stem Cell Bank (US), ES Tools (European Union), the Canadian Stem Cell Network, and many other initiatives, and he chairs the Membership Committee of the International Society for Stem Cell Research and is a member of its Finance Committee. He is on the Editorial Boards of *Cell Stem Cell*, *Stem Cells*, *Stem Cell Research*, and *PLoS1*. He is author of over 100 peer-reviewed publications and inventor on 14 issued patents and published patent applications.

Barry Rosen, PhD

Dr. Rosen is at the Wellcome Trust Sanger Institute (WTSI) in Cambridge, UK, an institute focused on engaging researchers in projects that seek to further

understanding of gene function in health and disease and to generate data and resources of lasting value to biomedical research. His Ph.D. work was with Dr. Bruce Spiegelman of Harvard University on the molecular biology of adipocytes and his post-doctoral work on mouse embryology with the late Dr. Rosa Beddington in Edinburgh. He then went on to work on gene trap mutagenesis strategies and mouse embryology in INSERM in Nice, France. In 2003 he joined the Sanger focusing on the development of high throughput approaches for the systematic mutagenesis of all genes in mouse embryonic stem cells as part of the EUCOMM and KOMP programs, which formed a major part of the International Knockout Mouse Consortium. He has been a Group Leader/Senior Scientific Manager in the ES Cell Mutagenesis Team for the past 7 years and one of the key scientists developing both wet lab and informatics based approaches to functional annotation of the mouse genome. These efforts have continued as part of the EU's EUCOMMTOOLS program where Dr. Rosen has managed a program to make 250 new strains of Cre driver mice. He also runs a laboratory that develops bespoke mouse models for the Sanger Faculty.

For the past several years he has also been involved in genome engineering and differentiation of human stem cells both at the Sanger and in collaboration with the Centre for Regenerative Medicine at Cambridge University (with Prof. Roger Pedersen) and the differentiation of human stem cells to adipocyte lineages (with Prof. Toni Vidal). Presently he is involved at the Sanger in the development of approaches to use designer nucleases (TALENs/CRISPRs) for large scale genome engineering of human stem cells.

Dr. Rosen is also active as an educator at the WTSI, serving as chief instructor for the past five years on an intensive course on Genome Manipulation of Mammalian Stem Cells and is well published on topics related to his areas of expertise and speaks at various international conferences on large scale mutagenesis strategies.

Steven Jon Russell, MD, PhD

Dr. Russell is Assistant Professor of Medicine at Harvard Medical School and an Attending Physician at Massachusetts General Hospital. Dr. Russell earned his MD and PhD at University of Texas Southwestern Medical School and did his Residency and Fellowship at Massachusetts General Hospital. He is certified by the American Board of Internal Medicine in Internal Medicine and Endocrinology, Diabetes & Metabolism.

Dr. Russell is a principal investigator in a collaborative group of investigators from Massachusetts General Hospital and Boston University who are working to make automated blood glucose control a reality. In order to reduce the impact of diabetes on those who live with this disorder, the group is developing a closed-loop artificial pancreas blood glucose control system capable of monitoring blood glucose levels every five minutes and utilizing a computer algorithm to deliver rapid-acting insulin and glucagon as needed to avoid hyper- and hypoglycemia. They have recently completed the first outpatient trial of this device. Other projects are focused on automated management of glucose in the hospital, insulin pharmacokinetics, and

real-time insulin sensing. Dr. Russell’s clinical interests include diabetes mellitus, hyperglycemia of critical illness, intensive insulin therapy, and new technology in the management of diabetes.

Dr. Russell is active in a number of professional societies and is a reviewer for several journals including *The New England Journal of Medicine*, *Diabetes*, *Diabetes Care*, *Aging Cell*, *The FASEB Journal*, and *Molecular and Cellular Endocrinology*. His research is supported by the National Institutes of Health (NIH), the Leona M. and Harry B. Helmsley Charitable Trust, the American Diabetes Association, and the Juvenile Diabetes Research Foundation.

Reappointment of Scientific Members to the Grants Working Group

Grants Working Group Members originally appointed in late 2006 and early 2007 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. We propose 2 and 6-year reappointment terms for this cohort as indicated in table below.

Proposed Reappointments to GWG

Last	First	Term (Yrs.)	Expertise
Heimfeld	Shelly	6	Cellular Therapy; Hematology; GMP Cell Production
Lemischka	Ihor	2	Cell Fate; Hematopoietic Stem Cells; Systems Biology; Stem Cell Biology
Zwaka	Thomas	6	Pluripotent Stem Cell Biology; Molecular Genetics

Shelly Heimfeld, Ph.D.

Dr. Heimfeld is a Full Faculty Member at the Fred Hutchinson Cancer Research Center in Seattle, Washington and serves as Scientific Laboratory Director for the Cellular Therapy Laboratory and cGMP Therapeutic Manufacturing Facilities. These facilities are responsible for all minimally and more extensively manipulated cell components used for treatment of patients at the Center. His primary responsibilities are to ensure the safety, quality, and effectiveness of each product, but also include implementation of new technologies, translation of basic science procedures into appropriate clinical protocols, product development, process improvement, and regulatory compliance.

Dr. Heimfeld received his Ph.D. in Cell Differentiation from the University of California, Irvine and completed postdoctoral studies with Dr. Irv Weissman at Stanford before going into industry to work 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 internationally recognized for his research in hematopoietic-derived stem cells and the development of cell processing technologies for improved cancer therapy. His long-term goals for this area are to identify better markers for characterization of stem and progenitor cells, to improve isolation technologies, and to develop *ex vivo* manipulation strategies that can enhance the therapeutic potential of these cells. He has also been involved in the clinical development of T-cell based immunotherapy for various diseases.

Dr. Heimfeld is a Past-President and Past-Chair of the Executive Advisory Board for ISCT (International Society of Cellular Therapy). He is a leading authority in regulations and lab practices needed for cell therapies, including Good Laboratory Practice (GLP), Good Tissue Practice (GTP), and Good Manufacturing Practice (GMP). Dr. Heimfeld continues to work with the Food and Drug Administration (FDA) to facilitate exchange of ideas in the rapidly evolving area of Cell Therapy.

Ihor R. Lemischka, PhD

Dr. Lemischka is the Director of The Black Family Stem Cell Institute, the Lillian and Henry M. Stratton Professor of Developmental and Regenerative Biology, and Professor of Pharmacology and Systems Therapeutics at Mount Sinai. Dr. Lemischka earned his PhD at Massachusetts Institute of Technology (MIT). There, he served as a postdoctoral research associate and also completed a fellowship at MIT's Center for Cancer Research. Dr. Lemischka then went to the Whitehead Institute for Biomedical Research in Cambridge, Massachusetts where he completed his postdoctoral training. In 1986, he joined the faculty at Princeton University where he rose from Assistant Professor to Professor of Molecular Biology. He remained on the Princeton faculty for 21 years before coming to Mount Sinai.

An internationally renowned stem cell biologist, Dr. Lemischka has patented techniques to isolate stem cells and has significantly advanced the study of stem cell activity and behavior. Dr. Lemischka is working to establish Mount Sinai as the leading stem cell institute in the United States, which he hopes will serve as a model worldwide.

Stem cell research has clinical implications for many diseases. The first step is to understand what makes the stem cell “decide” what type of cell it will become and how it communicates with neighboring cells. Dr. Lemischka hopes to characterize the stem cell's decision-making process and regulatory network, which will then help scientists manipulate stem cell decisions and develop therapies that could treat diseases.

A vocal advocate of stem cell research, Dr. Lemischka believes the latest findings about stem cells are just the tip of the iceberg of all the medical advances that will come from stem cell research. Dr. Lemischka stands by the notion that scientific freedom is key to resolving some of the biggest mysteries in medicine.

A member of the International Society for Stem Cell Research, he has traveled the world to educate the public about stem cell behavior and has delivered countless lectures about stem cell differentiation.

Thomas P. Zwaka, MD, PhD

Dr. Thomas Zwaka, an Assistant Professor in the Department of Molecular and Cellular Biology and the Center for Cell and Gene Therapy at the Baylor College of Medicine also serves as Director of the Baylor Embryonic Stem Cell Core, and was one of the founders of the Stem Cells and Regenerative Medicine Center. After receiving his M.D. Ph.D. degrees from the University of Ulm, Germany, Dr. Zwaka completed postdoctoral fellowships in molecular cardiology at the University of Ulm, and in human and mouse embryonic stem cell biology at the National Primate Research Center at the University of Wisconsin, Madison with Dr. James Thompson. He has received numerous awards and honors, including the Gillson Longenbaugh Foundation Junior Investigator award and the Lance Armstrong Foundation Junior Investigator Award. He has served on a number review panels for the National Institutes of Health (NIH), including the NIGMS Special Emphasis Panel for human embryonic stem cell research, and he sits on the Scientific Advisory Board of the Genetics Policy Institute and Stem Cells Source, Inc.

Dr. Zwaka's research currently focuses on fundamental questions surrounding human embryonic stem cell biology, including how to reverse the process of differentiation and "re-program" any given cell type into a pluripotent stem cell. Dr. Zwaka has authored fundamental publications on the genetic modification of human embryonic stem cells, and has filed patents on embryonic stem cell differentiation and modification both in the US and internationally.