CIRM

CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

STEM CELL RESEARCH: CHARTING NEW DIRECTIONS FOR CALIFORNIA

2005

October 1–2, 2005, Renaissance Parc 55, San Francisco

CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

WELCOME FROM THE PRESIDENT

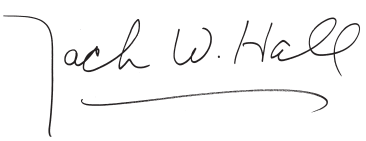
Dear Colleagues,

Stem Cell Research: Charting New Directions for California marks a major milestone for the California Institute for Regenerative Medicine (CIRM). As we embark on our ambitious project, based on Proposition 71, of providing significant and continuous funding for stem cell research in California, we need to chart a scientific course that will lead us along the surest pathway to our goal, which is to provide safe and effective therapies for those with disease and disability.

This meeting is the first step in that process. We have invited leading investigators from all over the world to help us survey the changing scientific terrain of stem cell research so that we can find the best and quickest path — and there may be more than one — to achieve our aims. We look not only to our speakers, but to our attendees for discussion aimed to set out a series of scientific priorities for CIRM that will materially accelerate progress in stem cell research. As part of this discussion, we welcome to the meeting scientists from both non-profit and for-profit research organizations in California, patient advocates and members of the public. To reach the broadest possible audience, we have arranged for the meeting to be webcast in real-time.

I anticipate an exciting two days of vigorous scientific discussion about the best ways to propel stem cell research in California toward achieving our aim. We look forward to your comments and participation.

Sincerely,



Zach W. Hall, Ph.D.
President, CIRM

PROGRAM

Friday, September 30, 2005

6:00 – 8:00 pm Reception for all attendees sponsored by California Healthcare Institute
The Westin St. Francis Hotel, Victor's and Alexandra's, 32nd floor

Saturday, October 1, 2005

Renaissance Parc 55, Parc Ballroom, 4th floor

8:30 – 9:00 am **Welcome**
Zach Hall, Ph.D., President, CIRM
Robert Klein, Chairman, ICOC

9:00 – 11:00 am **SESSION I**
Cellular Therapeutics: Clinical State of the Art and Challenges for the Future

Chairs: George Daley, M.D., Ph.D., Harvard Medical School
Paul Berg, Ph.D., Stanford University

Speakers: Rob Negrin, M.D., Stanford University
Hematopoietic Stem Cell Transplantation: Where We Have Been and Where We Are Going

Jeff Bluestone, Ph.D., University of California, San Francisco
A Cure for Type I Diabetes

Olle Lindvall, M.D., Ph.D., University of Lund, Sweden
Stem Cell Therapy for Human Brain Disease: How Can We Make It Work?

11:00 – 11:15 am **Break** — Pick up box lunches

11:15 – 1:15 pm **SESSION II**
Stem Cells and Therapies: Lessons from the FDA and Industry

Chairs: Edward Penhoet, Ph.D., Vice Chair, ICOC
Tina Nova, Ph.D., Member, ICOC

Speakers:	Jane Lebkowski, Ph.D., Geron Corporation *Preclinical Studies to Support Clinical Testing of Stem Cell-Based Therapies*
	Alan Smith, Ph.D., Cognate Therapeutics, Inc. *GMP Banking, Manufacturing and Quality Control of Stem Cells for Clinical Use*
	Donald Fink, Ph.D., Food and Drug Administration *Developing Stem Cell-Based Therapies: Engaging the FDA*
1:15 – 3:15 pm	**SESSION III** **Stem Cells as Tools for Disease Research and Therapy**
Chairs:	Fred Gage, Ph.D., The Salk Institute Mary Maxon, Ph.D., Deputy Vice Chair, ICOC
Speakers:	Rudolph Jaenisch, Ph.D., The Whitehead Institute, MIT *SCNT for Understanding Disease*
	Inder Verma, Ph.D., The Salk Institute *Molecular Manipulation of Embryonic Stem Cells*
	Peter Schultz, Ph.D.,The Scripps Research Institute *Small Molecules and High-Throughput Screening in Embryonic Stem Cells*
3:15 – 3:30 pm	**Break**
3:30 – 5:30 pm	**SESSION IV** **Self-Renewal of Stem Cells**
Chairs:	Peter Andrews, Ph.D., University of Sheffield, United Kingdom Janet Wright, M.D., Member, ICOC
Speakers:	Haifan Lin, Ph.D., Duke University School of Medicine *Understanding Stem Cell Self-Renewal: Progress and Prospect*
	Andras Nagy, Ph.D., Mount Sinai Hospital, Toronto, Canada *Development of Tools for Studying hESC Self-Renewal*
	Martin Pera, Ph.D., The Monash Institute of Medical Research, Australia *Development of Conditions for Defined Culture and Scale-Up*

7:00 – 10:00 pm Dinner for session chairs, speakers, ICOC members, and confirmed dinner attendees, supported by the Alliance for Stem Cell Research.
Renaissance Parc 55, Barcelona II, 3rd floor

Speaker: Brad Margus, CEO and co-founder, Perlegen Sciences, Inc.
A Father's Perspective: Science and Stem Cells, Hype and Hope

Sunday, October 2, 2005

Renaissance Parc 55, Parc Ballroom, 4th floor

8:00 – 10:00 am **SESSION V**
Fate Decisions – Good and Bad Choices

Chairs: Owen Witte, M.D., University of California, Los Angeles
Irving Weissman, M.D., Stanford University

Speakers: Nissim Benvenisty, M.D., Ph.D., Hebrew University of Jerusalem, Israel
How Can We Control Differentiation of ES Cells?

Irving Weissman, M.D., Stanford University
Current Status of in vivo Systems to Monitor Stem Cell Differentiation and Function

Michael Clarke, M.D., University of Michigan
Bad Stem Cell Choices in Cancer Development

10:00 – 10:15 am **Break**

10:15 – 12:15 pm **SESSION VI**
Bridging the Gap Between Bench and Bedside

Chairs: Ian Duncan, Ph.D., University of Wisconsin
Arlene Chiu, Ph.D., CIRM

Speakers: David Scadden, M.D., Harvard Medical School
The Stem Cell Niche: A Drugable Target?

Jon Odorico, M.D., University of Wisconsin
Hurdles to Overcome in Applying hES Cells for Treatment of Diabetes

Hans Keirstead, Ph.D., University of California, Irvine
Human Embryonic Stem Cell-Derived Oligodendrocyte Progenitors: A Defined Cell Population for a Defined Need

12:15 – 12:30 pm **Break** — Pick up box lunches

12:30 – 2:30 pm **Review of Scientific Sessions**
Gil Sambrano, Ph.D., CIRM
Theo Palmer, Ph.D., Stanford University
Julie Baker, Ph.D., Stanford University

2:30 – 2:45 pm **Break**

2:45 – 6:00 pm **Summary of Meeting**
- Recommendations to CIRM presented by Chairs from each Session
- Closing remarks by Zach Hall, Ph.D., President, CIRM

CHAIRS & SPEAKERS

Peter W. Andrews, D.Phil.
University of Sheffield, United Kingdom

Dr. Andrews is the Arthur Jackson Professor of Biomedical Science and former chairman of the Department of Biomedical Science at the University of Sheffield. In addition, he co-founded and serves as director of Axordia Ltd., a company established to develop commercial applications of stem cell biology. Dr. Andrews received a B.S. from the University of Leeds, a D.Phil. from Oxford University, and an M.B.A. from the University of Pennsylvania's Wharton School. A leader in developing infrastructure to support collaborative stem cell research, he coordinates the International Stem Cell Initiative and has organized an annual U.K. human embryonic stem cell training course. He is also a member of the User Liaison and Management Committees of U.K.'s Stem Cells Bank. Dr. Andrews' own research interests focus upon the biology of human embryonic carcinoma cells and human embryonic stem cells.

Julie Baker, Ph.D.
Stanford University, Palo Alto, CA

Dr. Baker's research interest focuses on understanding the genetic program of cell fate specification within the early mammalian embryo. This interest was kindled during her doctoral research at Columbia University, where she studied insulin-like growth factor signaling in the mammalian embryo. During her postdoctoral fellowship at the University of California, Berkeley, she developed an expression-cloning screen using amphibian embryos to identify mammalian cell fate molecules. Currently, as an assistant professor in the Genetics Department at Stanford University, she heads a laboratory that is dissecting the pathways involved in endoderm formation and has been using the knowledge gained to steer human embryonic stem cells along endodermal lineages.

Nissim Benvenisty, M.D., Ph.D.
Hebrew University of Jerusalem, Israel

Dr. Benvenisty received his M.D. (1983) and Ph.D. (1986) in developmental biochemistry from Hebrew University of Jerusalem, Israel, and is currently a professor in the Department of Genetics and the Herbert Cohn Chair in Cancer Research at the Hebrew University. In 1999, he was a visiting professor in the Department of Genetics at Harvard University. Among the numerous distinctions that he has received are the Teva Prize for excellent research on stem cells in 2003 and the Hestrin Prize in Biochemistry and Molecular Biology. Dr. Benvenisty is a member of the International Stem Cells Initiative Steering Committee.

Paul Berg, Ph.D.

Stanford University, Palo Alto, CA

Dr. Berg, the Robert W. and Vivian K. Cahill Professor in Biochemistry and Cancer Research and professor emeritus at Stanford University, is a renowned pioneer of the gene-splicing field. He received his B.S. from Pennsylvania State University (1948) and his Ph.D. in biochemistry from Case Western Reserve University (1952). After postdoctoral work in Copenhagen, Denmark he joined the faculty of Washington University Medical School (1954). In 1959, he moved to Stanford University where he served as executive head of the Department of Biochemistry from 1969 to 1974. Professor Berg has received international recognition and many prestigious awards for his work on the genetic mechanisms through which cells form proteins. Most notably, Dr. Berg was awarded the Lasker Basic Science Award and the Nobel Prize in Chemistry for developing methods to map the structure and function of DNA.

Jeffrey Bluestone, Ph.D.

University of California, San Francisco, CA

Dr. Bluestone is the A.W. and Mary Margaret Clausen Distinguished Professor and Director of the Diabetes Center at the University of California, San Francisco (UCSF). He currently serves as director of the Juvenile Diabetes Research Foundation Collaborative Center for Cellular Therapy and was interim director (2002-2004) of UCSF's Developmental and Stem Cell Biology Program. A graduate of Rutgers University (B.S., M.S.) and Cornell University (Ph.D.), Dr. Bluestone has received numerous awards for his research and is internationally recognized as an expert on diabetes and immune system research, in particular for his contributions toward clarifying the biological basis of immune tolerance. He is perhaps best known for studies on molecular-level approaches to control the immune activity of antibodies and efforts to boost the beneficial effects of tolerance-inducing drugs. This research has stimulated recent progress in the use of islet cell transplantation to treat Type 1 diabetes.

Arlene Y. Chiu, Ph.D.

California Institute for Regenerative Medicine

Dr. Chiu, director of scientific program and review activities at CIRM, served as associate director of the Office of Research Administration of the National Institute of Biomedical Imaging and Bioengineering at the National Institutes of Health (NIH). Prior to that, she was the program director for Stem Cell Research and for research on Spinal Cord Injury at the National Institute of Neurological Disorders and Stroke. Dr. Chiu served on the NIH Stem Cell Task Force and the NIH Stem Cell Implementation Committee, organized workshops on stem cells and led efforts to promote cooperation with the U.S. Food and Drug Administration in expediting the use of stem cells in therapies. In 2004, she received the NIH Director's Award for her outstanding contributions to the development of stem cell research. Dr. Chiu graduated *summa cum laude* from Stanford University and she received pre-doctoral training at the California Institute of Technology and postdoctoral training at Washington University, St. Louis. As an independent investigator, her research focused on mammalian motor neurons and their responses to injury and disease.

Michael Clarke, M.D.

Stanford University, Palo Alto, CA

Dr. Clarke is deputy director of the Cancer Stem Cell Institute and Professor of Internal Medicine at Stanford University. After receiving his medical degree from Indiana University he specialized in general oncology with a main focus on blood and marrow transplantation. Dr. Clarke's research focuses on the molecular regulation of hematopoiesis and hematopoietic transformation, gene therapy and the molecular mechanism of malignant transformation. His laboratory was the first to identify a molecular pathway that regulates self-renewal of adult stem cells, and has developed methods to prospectively identify a "cancer stem cell" population in breast cancer. These findings link the process of self-renewal in normal stem cells to cancer and offers implications for the treatment and diagnosis of human cancers.

George Q. Daley, M.D., Ph.D.

Harvard Medical School, Boston, MA

Dr. Daley is an associate professor of Biological Chemistry and Pediatrics at Harvard Medical School. He received his bachelor's degree *magna cum laude* from Harvard University (1982), and a Ph.D. in biology from MIT (1989), working with Nobel laureate David Baltimore. Dr. Daley also earned an M.D. from Harvard Medical School and the Harvard-MIT Division of Health Sciences and Technology. Among his many awards, Dr. Daley is a recipient of the NIH Director's Pioneer Award, which provides a five-year unrestricted grant to pursue highly innovative research. Dr. Daley's laboratory (together with Dr. Rudolf Jaenisch) reported the first successful application of therapeutic cloning of embryonic stem cells to treat a genetic disease in a mouse model of immune deficiency, and the first creation of functional sperm cells from embryonic stem cells, work that was cited by *Science* magazine as a "Top Ten" breakthrough for 2003. Dr. Daley's research is aimed at translating insights in stem cell biology into cellular therapies for degenerative, malignant, and genetic diseases.

Ian Duncan, Ph.D.

University of Wisconsin, Madison, WI

Dr. Duncan is professor of neurology in the Department of Medical Sciences at the University of Wisconsin, Madison, School of Veterinary Medicine. He received his doctorate in neuropathology from Glasgow University in the United Kingdom and completed a postdoctoral fellowship in experimental medicine at McGill University in Montreal, Canada. Dr. Duncan has received significant recognition for his research contributions, including election as a fellow of the Royal College of Veterinary Surgeons and of the Royal College of Pathologists for meritorious contributions to the literature. His research focuses on the use of embryonic stem cell-derived progenitors in brain repair. Dr. Duncan's main interest is to investigate the potential of glial cell transplantation as a therapeutic approach to repair the demyelinated or non-myelinated areas of the central nervous system.

Donald W. Fink, Jr., Ph.D.

Food and Drug Administration, Center for Biologics Evaluation and Research, Rockville, MD

Dr. Fink is a regulatory review scientist at the FDA, in CBER's Office of Cellular, Tissue and Gene Therapies, where he coordinates CBER's human embryonic stem cell review team and serves as the FDA liaison to the NIH Stem Cell Task Force. Dr. Fink received his B.A. from Northwestern University and his Ph.D. in pharmacology from the University of Minnesota. With over 10 years of experience evaluating Investigative New Drug applications and compiling a review portfolio of biologic products that includes recombinant therapeutic proteins, monoclonal antibodies, therapeutic vaccines, and cellular therapies, Dr. Fink recently has been actively involved in regulatory matters pertaining to biologic products comprised of stem cells. He has organized an FDA advisory committee meeting about the use of stem cells in cellular replacement therapies for neurological disorders, and co-founded and co-chaired (with Dr. Arlene Chiu) an FDA-NIH interagency cell and gene therapy working group. Dr. Fink recently completed a detail appointment in the FDA's Office of Combination Products and is a member of the Tissue Reference Group.

Fred H. Gage, Ph.D.

Salk Institute, La Jolla, CA

Dr. Gage is a professor of Laboratory Genetics at the Salk Institute for Biological Studies. Known for his discovery of structural and functional plasticity in the adult mammalian brain, Dr. Gage has shown that adults continue to generate new neurons throughout life, and that birth and survival of the neurons is regulated by behavior. He also demonstrated that neurotrophic factors can induce functional repair of the damaged and aged brain. Dr. Gage earned his B.S. from the University of Florida and his Ph.D. from Johns Hopkins University. He has received broad recognition for his research, including the Charles A. Dana Award for Pioneering Achievements in Health and Education, the Christopher Reeve Research Medal, and the Max Planck Research Prize. He is past president of the Society for Neuroscience and a member of the National Academy of Sciences.

Zach W. Hall, Ph.D.

California Institute for Regenerative Medicine

Dr. Hall is president of the CIRM, established by Proposition 71 to promote stem cell research in California. A former director of the National Institute of Neurological Disorders and Stroke, Dr. Hall recently served as the senior associate dean at the Keck School of Medicine of the University of Southern California, and as executive vice-chancellor of the University of California, San Francisco, where he led planning for the new Mission Bay campus. He has published over 100 original articles and was a founding editor of *Neuron*, a leading journal in the field of neuroscience. In 2003, he received the Purkynje Medal for Scientific Achievement from the Czech Academy of Science. An English major at Yale University, Dr. Hall received his Ph.D. from Harvard University followed by a postdoctoral fellowship at Stanford University. He was a faculty member at Harvard Medical School and later at UCSF.

Rudolph Jaenisch, M.D.

Massachusetts Institute of Technology, Cambridge, MA

Dr. Jaenisch is a professor of biology at the Massachusetts Institute of Technology and founding member of the Whitehead Institute for Biomedical Research in Cambridge, MA. After completing his thesis in molecular biology at the Max Planck Institute for Biochemistry in Munich, he received postdoctoral training at Princeton University and at the Salk Institute in molecular virology before moving to his current position at the Whitehead Institute. Dr. Jaenisch is a pioneer in the use of transgenic mice; his work has led to important advances in understanding cancer, neurological and connective tissue diseases, and developmental abnormalities. Mice from his laboratory have been used to explore basic questions such as the role of DNA modification, genomic imprinting, X chromosome inactivation, and most recently, the nature of stem cells, epigenetic reprogramming and somatic cell nuclear transfer. The long-range goals of his laboratory are to understand epigenetic regulation of gene expression in mammalian development and disease.
Dr. Jaenisch has coauthored more than 300 research papers and has received numerous prizes and recognition, including an appointment to the National Academy of Sciences in 2003.

Hans Keirstead, Ph.D.

University of California, Irvine, CA

Dr. Keirstead is an associate professor at the Reeve-Irvine Research Center, a leading center for spinal cord injury research, at the University of California at Irvine. His thesis research, conducted at the University of British Columbia in Vancouver, Canada, led to a novel method for regeneration in the damaged spinal cord and formed the basis of several patents and the formation of a company in 1999 to bring this treatment toward clinical trials. For these achievements, he received the Cameron Award for outstanding Ph.D. thesis in Canada. As a postdoctoral fellow at the University of Cambridge, he was elected to two senior academic posts: Fellow of the Governing Body of Downing College, and Senate Member of the University of Cambridge. Dr. Keirstead was recently awarded the Distinguished Assistant Professor Award, the UCI Academic Senate's highest honor. The focus of his research is the development of strategies to limit degeneration and enhance regeneration after spinal cord injury, and his laboratory has developed human reagents necessary for clinical trials.

Robert Klein, J.D.

Independent Citizens' Oversight Committee, CIRM

As the creator and primary supporter of Proposition 71, which led to the establishment of the CIRM, Mr. Klein has been instrumental in advocating for research and the development of cures for a variety of conditions, including Alzheimer's Disease and diabetes. In 2005 he was honored by *Time* magazine as one of the "100 Most Influential People of the Year." He currently serves as chair of the Independent Citizen's Oversight Committee of the CIRM. As president of Klein Financial Corporation, a property investment banking consulting company, Mr. Klein is known for his many accomplishments in the real estate world such as authoring the California Housing Finance Agency Act, developing California's first tax credit National Historic Site Restoration Project, and creating California's first local governmental, tax-exempt, bond-financed, affordable apartment project. In addition to his work in real estate, Mr. Klein continues to be involved in health initiatives outside of CIRM. He serves on the board of the Juvenile Diabetes Research Foundation. He received a juris doctor's degree from Stanford Law School and a bachelor's degree in history from Stanford University.

Jane S. Lebkowski, Ph.D.
Geron Corporation, Menlo Park, CA

Jane Lebkowski received her Ph.D. in biochemistry from Princeton University in 1982. Following a postdoctoral fellowship in the Department of Genetics, Stanford University, she joined Applied Immune Sciences in 1986, where she served as vice-president of Research and Development. When Applied Immune Sciences was acquired by Rhone Poulenc Rorer (RPR), Dr. Lebkowski remained at RPR (currently Sanofi-Aventis) as the vice-president of Discovery Research. In that position she coordinated preclinical investigations of gene therapy approaches for the treatment of cancer, cardiovascular disease and disorders of the nervous system, and directed the vector development, formulation, and delivery programs. In 1998, Dr. Lebkowski joined Geron Corporation as the senior director of Cell and Gene Therapies, and is currently senior vice-president of Regenerative Medicine. Dr. Lebkowski heads the human embryonic stem cell program at Geron, and coordinates both preclinical research and product development activities.

Haifan Lin, Ph.D.
Duke University, Durham, NC

Dr. Lin is an associate professor in the Department of Cell Biology and the founding co-director of the Duke University Stem Cell Research Program. He received his B.S. in biochemistry from Fudan University in Shanghai, China (1982), and his Ph.D. from Cornell University (1990), where he studied maternal control of embryonic mitosis in *Drosophila*. For his thesis research, Dr. Lin received Honorary Mention (second place) for the Larry Sandler Award by the Genetics Society of America (1990). He was awarded a Jane Coffin Child Fellowship for his postdoctoral training at the Carnegie Institution of Washington, where he studied oocyte determination mechanism in *Drosophila*, and pioneered the use of the *Drosophila* germline stem cell as a model for stem cell research. Dr. Lin's accomplishments in stem cell research include the discovery of the *piwi/argonaute* gene family, presently the only known gene family that regulates stem cell self-renewal – a function that is highly conserved during evolution in both animal and plant kingdoms. Dr. Lin has received several prestigious awards for his scientific contributions, is a founding officer of the International Society for Stem Cell Research and a member of the editorial board of the journal *Stem Cells*.

Olle Lindvall, M.D., Ph.D.
University of Lund, Sweden

Dr. Lindvall is the chairman of the Department of Clinical Neuroscience, professor of neurology, and head of the Section of Restorative Neurology and the Clinical Neurotransplantation Program at the University of Lund in Sweden. He received his Ph.D. (1974) and M.D. (1978) from the University of Lund and has distinguished himself as a world-renowned neurologist and researcher in gene and cell therapy for Parkinson's disease. Among the many awards that Dr. Lindvall has received are the Jubilee Prize from the Swedish Society of Medicine, an honorary medal from the Swedish Parkinson Association, and the Soderberg Prize from the Swedish Society of Medicine. Dr. Lindvall is an elected member of the board of the Swedish Research Council's medical division and served for four years as the chairman of the Swedish Movement Disorder Society.

Brad Margus

Perlegen Sciences, Mountain View, CA

Mr. Margus earned an M.B.A. from Harvard University and spent 14 years running a Florida-based international food company. After learning that two of his sons had the genetic neurodegenerative disease ataxia-telangiectasia (A-T), Mr. Margus formed a non-profit organization, the A-T Children's Project, to accelerate the pace of research on A-T. By recruiting outstanding scientists, organizing scientific conferences, orchestrating biomedical research projects, establishing a clinical center, funding clinical trials, and creating brain tissue and DNA banks, Mr. Margus' foundation has made significant progress on understanding A-T. Mr. Margus also became an advocate for patients with other rare genetic disorders and frequently testified before Congress on their behalf. In late 2000, he co-founded Perlegen Sciences, a California-based genetics company that works with non-profit foundations as well as major pharmaceutical industry partners to discover the genetic bases of diseases and predictive markers of drug response. Mr. Margus has served on institutional review boards, NIH advisory committees, and corporate boards. In addition to serving as volunteer president of the A-T Children's Project, Mr. Margus sits on the board of the Children's Neurobiological Solutions Foundation, which focuses on brain repair and regeneration.

Mary E. Maxon, Ph.D.

Independent Citizens' Oversight Committee, CIRM

Dr. Maxon is the deputy vice chair to Vice Chair Edward Penhoet of the ICOC. She received a Ph.D. from the University of California, Berkeley in molecular cell biology, where she studied human gene expression. Dr. Maxon studied genetics as a postdoctoral researcher at the University of California, San Francisco as a Helen Hay Whitney Fellow and began her career in biotechnology at Microbia, Inc. in Cambridge, MA. Recently, she was associate director and program leader at Cytokinetics, Inc. where she led the infectious diseases drug discovery program. Earlier this year, Dr. Maxon organized a conference in India in conjunction with the National Academy of Sciences on drug discovery opportunities for neglected diseases of the developing world and remains active in the global health community.

Andras Nagy, Ph.D.

Mount Sinai Hospital, Toronto, Canada

Dr. Nagy is a professor in the Department of Medical Genetics and Microbiology at the University of Toronto and a senior investigator in the Program of Development and Fetal Health at the Samuel Lunenfeld Institute. He completed a B.A. and an M.A. in mathematics and received a Ph.D. in genetics from the Lorand Eotvos University in Budapest, Hungary. Dr. Nagy currently holds the Canadian Institute of Health Records Senior Scientist Award, and, in partnership with Bristol-Myers Squibb, he was awarded the Medical Research Council of Canada/Pharmaceutical Manufacturers Association of Canada Scientist Award. His research interest is focused on the use of mouse genetics to study mammalian development and to apply this knowledge to human disease.

Robert S. Negrin, M.D.

Stanford University, Palo Alto, CA

Dr. Negrin is a professor of medicine at Stanford University, chief of the Division of Blood and Marrow Transplantation, and medical director of the Clinical Translational Laboratory. He received his undergraduate degree from the University of California, Berkeley and an M.D. from Harvard University. He then trained in internal medicine and hematology at Stanford University and joined the Stanford faculty in 1990. Dr. Negrin has authored over 125 manuscripts and 35 book chapters on basic and clinical transplantation immunology and hematopoietic cell transplantation. He has served as the president of the International Society of Cellular Therapy and is the president-elect of the American Society of Blood and Marrow Transplantation.

Tina S. Nova, Ph.D.

Independent Citizens' Oversight Committee, CIRM

Dr. Tina S. Nova serves as a representative from a commercial life science entity on the Independent Citizens' Oversight Committee, the governing body of the California Institute for Regenerative Medicine. She is the winner of the Athena Pinnacle Award and the Soroptimist Woman of Distinction Award for her renowned research work in biotechnology. Her extensive career has led to numerous patents, the co-founding of four life science companies in the San Diego biotechnology community, and service as publisher of several prestigious scientific journals. Dr. Nova received a Ph.D. in biochemistry from the University of California, Riverside and a bachelor's degree in biological sciences at the University of California, Irvine.

Jon S. Odorico, M.D.

University of Wisconsin, Madison, WI

Dr. Odorico is director of the Islet Cell Transplantation Program and associate professor in the Department of Surgery, Division of Organ Transplantation, at the University of Wisconsin. He also is a research associate at the WiCell Institute in Wisconsin. Dr. Odorico received his B.S. in Chemistry from Duke University, an M.D. from New York University, and completed his residency in general surgery as well as a postdoctoral research fellowship studying islet transplantation and thymic tolerance at the University of Pennsylvania in Philadelphia. Dr. Odorico has an active research laboratory that focuses on islet differentiation from embryonic stem cells.

Theo Palmer, Ph.D.

Stanford University, Palo Alto, CA

Dr. Palmer is an assistant professor of neurosurgery in the Program in Neurosciences at Stanford University. After earning his Ph.D. in experimental pathology from the University of Washington in Seattle, he trained as a postdoctoral fellow with Dr. Fred Gage at the Salk Institute, where his studies led to the identification and characterization of stem cells that continuously generate new neurons within the adult mammalian brain. At Stanford, Dr. Palmer's laboratory focuses on local and systemic signaling that regulates neural stem cells in the adult. His primary goal is to understand signaling environments that specifically support the production of replacement neurons, both as a natural process within the brain as well as via transplanted stem cell populations to treat injury or disease.

Edward E. Penhoet, Ph.D.

Independent Citizens' Oversight Committee, CIRM

Dr. Penhoet earned his A.B. in biology from Stanford University in 1963, and his Ph.D. in biochemistry from the University of Washington in 1968. He is currently the president of the Gordon and Betty Moore Foundation in San Francisco and has served as dean and professor of the School of Public Health and professor of Molecular and Cell Biology at the University of California, Berkeley. Since 2000, he has been a director at Alta Partners, a pioneering venture capital firm that focuses on early-stage investing in life sciences, information technology and communications. He is also a co-founder and director of Chiron Corporation, one of the world's leading biotechnology companies. From the company's founding in 1981 until April 30, 1998, Dr. Penhoet served as Chiron's president and chief executive officer. Dr. Penhoet is vice chairman of the Independent Citizens' Oversight Committee, the governing body of the California Institute for Regenerative Medicine.

Martin Pera, Ph.D.

Monash Institute of Medical Research, Clayton, Australia

Dr. Pera is a 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 received a B.A. in English language and literature from the College of William and Mary, and a Ph.D. in pharmacology from George Washington University. Dr. Pera trained as a postdoctoral fellow 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. His research interests include the cell biology of human pluripotent stem cells, early human development, and germ cell tumors. Dr. Pera was among a small number of researchers who pioneered the isolation and characterization of pluripotent stem cells from human germ cell tumors of the testis, which provided an important framework for the development of human embryonic stem cells. His laboratory at Monash University was the second in the world to isolate embryonic stem cells 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 embryonic stem cell research, and is a member of the Steering Group of the International Stem Cell Initiative.

Gilberto R. Sambrano, Ph.D.

California Institute for Regenerative Medicine

Dr. Sambrano is the first scientific review officer to join CIRM. His scientific education includes a B.S. in biology from the University of Texas at El Paso and a Ph.D. in biomedical sciences from the University of California, San Diego. He trained as a postdoctoral fellow with the Cardiovascular Research Institute at the University of California, San Francisco, where he later accepted a position of assistant professor in the Department of Cellular and Molecular Pharmacology. In 2001, Dr. Sambrano took on a notable position to coordinate efforts of the Alliance for Cellular Signaling, a multi-institutional and multi-disciplinary consortium whose goal is to understand the basic principles that regulate the complex network of chemical signals within cells.

David Scadden, M.D.

Harvard Medical School, Boston, MA

Dr. Scadden, a professor of medicine at Harvard Medical School, is director of the MGH Center for Regenerative Medicine and Technology. He is also co-director of the Harvard Stem Cell Institute and chief of the Hematologic Malignancies Program at the MGH Cancer Center. Dr. Scadden is a board certified hematologist/oncologist having received his training at the Brigham and Women's Hospital and the Dana-Farber Cancer Institute. His research interest is in adult stem cell biology, focusing on reconstituting immune function using the hematopoietic stem cell in settings of cancer and AIDS. He directs both a basic research laboratory and a translational clinical research program. His basic research has defined molecular regulators constraining hematopoietic stem cell proliferation and identifying critical components of the microenvironment in which stem cells reside. Dr. Scadden serves on the executive committee of the Harvard Medical School Division of AIDS and is a member of the National Cancer Institute Board of Scientific Counselors at the National Institutes of Health.

Peter G. Schultz, Ph.D.

The Scripps Research Institute, La Jolla, CA

Dr. Schultz is currently the Scripps Professor of Chemistry at the Scripps Research Institute and director of the Genomics Institute of the Novartis Research Foundation in La Jolla, CA. He received both his bachelor and doctoral degrees from the California Institute of Technology. His work, which spans the interface of chemistry, biology, and materials science, has brought him distinction and recognition through numerous awards. Most notably, Dr. Schultz was elected to the National Academy of Sciences, USA (1993) and the Institute of Medicine of the National Academy of Sciences (1998). He has also founded several biotechnology companies including Affymax Research Institute, Symyx Technologies, Syrrx, Kalypsys, Phenomix, Ilypsa, and Ambrx.

Alan K. Smith, Ph.D.

Cognate Therapeutics, Inc., Baltimore, MD

Dr. Smith earned a Ph.D. in biochemistry from Utah State University and is currently president and chief operating officer for Cognate Therapeutics, Inc. where he oversees general management, research and development, preclinical, regulatory, manufacturing, quality control and clinical functions. From 2000 to 2002, prior to joining Cognate, Dr. Smith served as chief operating officer and senior vice-president of Research and Development at Osiris Therapeutics, a company with three stem cell products for oncology, cardiology, and orthopedics currently in clinical trials. Dr. Smith has also held senior management roles in several other cell therapy companies, including Aastrom Biosciences, Inc. and Geneic Sciences. Dr. Smith also serves on the board of directors of Chata Biosystems of Fort Collins, CO and MGP Biotechnologies of Irvine, CA.

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Dr. Verma is one of the world's leading authorities on the development of viruses for gene therapy vectors. He received his Ph.D. in biochemistry at the Weizmann Institute of Science in Israel and completed his postdoctoral work at MIT. He is a professor in the Laboratory of Genetics at the Salk Institute and was named the American Cancer Society Professor of Molecular Biology. His laboratory has two principal aims: 1) understanding the molecular mechanism of the function of proto-oncogenes and suppressor genes, and 2) gene therapy. Dr. Verma has received broad recognition for his research contributions including election to the National Academy of Sciences, the Institute of Medicine of the National Academy of Sciences, and American Academy of Arts and Sciences.

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Dr. Weissman is a professor in the departments of Pathology, Developmental Biology, and Neurosurgery at Stanford University. He is also the director of the Institute for Cancer and Stem Cell Biology and Medicine. He has co-founded several companies, including SyStemix in 1988, StemCells in 1996, and Celtrans (now Cellerant), the successor to SyStemix, in 2001. Dr. Weissman's research focuses on the biology and evolution of stem cells and progenitor cells, mainly from blood and brain. His laboratory was the first to identify and isolate blood-forming stem cells from mice. Dr. Weissman pioneered the study of the genes and proteins involved in cell adhesion events required for lymphocyte homing to lymphoid organs *in vivo*, either as a normal function or as events involved in malignant leukemic metastases. He is the recipient of numerous awards for his research contributions, and is a member of the National Academy of Sciences, the Institute of Medicine at the National Academy, and the American Association of Arts and Sciences.

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Dr. Witte received his undergraduate degree from Cornell University and his M.D. from Stanford University. He completed postdoctoral research at MIT then joined the faculty at UCLA, where he is presently an investigator of the Howard Hughes Medical Institute. He is also a professor in the department of Microbiology, Immunology and Molecular Genetics and the department of Molecular and Medical Pharmacology at the David Geffen School of Medicine at UCLA. In 2005, he was named the Founding Director of the Institute of Stem Cell Biology and Medicine. Dr. Witte has made significant contributions to the understanding of human leukemias, immune disorders, and epithelial cancer stem cells. He is a member of the National Academy of Sciences, the American Academy of Arts and Sciences, and was recently elected to the Institute of Medicine.

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Alliance For Stem Cell Research

The mission of the **Alliance for Stem Cell Research** is to advance the field of stem cell research and regenerative medicine, and to educate the public about its potential and progress. CIRM thanks the Alliance for helping to support Saturday night's dinner. For more information about the Alliance for Stem Cell Research, visit: *www.allianceforstemcellresearch.org.*

BAYBIO

BayBio is Northern California's life science association, supporting the life science community through advocacy, enterprise support, best practice dissemination and enhancement of research collaboration. Membership includes organizations engaged in or supportive of research, development, and commercialization of life science technologies. CIRM wishes to thank BayBio for assisting in the underwriting of the webcasting of our program: *Stem Cell Research: Charting New Directions for California*. For more information about BayBio, visit: *www.baybio.org.*

C - H - I CALIFORNIA HEALTHCARE INSTITUTE

The **California Healthcare Institute** (CHI), represents California's life sciences community of leading research institutions and universities, biotechnology, pharmaceutical, medical device, and diagnostics firms. CIRM wishes to thank CHI for cocktails, hors d'ouevres and spectacular views of San Francisco at the welcome reception on Friday night. For more information about CHI, visit: *www.chi.org.*

mosaic
event management

CIRM wishes to thank **Mosaic Event Management** for their excellent services in organizing and coordinating this conference. For more information about Mosaic, visit: *www.mosaicevents.com*

thesciencenetwork

The Science Network (TSN) is a visionary venture by a coalition of world-renowned scientists and media professionals to build a multi-media programming platform that will be a trusted destination for those concerned with science, particularly its impact on society. Part of its core mission is to provide unfiltered discussion of science issues that intersect with social policy. CIRM wishes to thank TSN for their participation in bringing this conference to the public via webcast. For more information about TSN, visit: *www.csntv.org*

VitalVenture Networks

Vital Venture Networks (VVN) is a community-builder and a conference management group that designs and manages events for leading investors, executives, markets and institutions involved in the life sciences. VVN also has experience in working with the financial markets — both the capital and venture markets — and leading biomedical research institutions and academies. CIRM wishes to thank VVN for their help in organizing this meeting. For more information about VVN, contact: *mwhitehouse@optonline.net*