

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

Mickie Bhatia, PhD

Dr. Mickie Bhatia received his B.Sc. (Honors) in Molecular Biology at McMaster University, Hamilton; and his Ph.D. in Human Biology and Nutritional Sciences at the University of Guelph, Canada.

Dr. Bhatia is the Scientific Director of the Stem Cell and Cancer Research Institute (SCC-RI), appointed Chair in Stem Cell and Cancer Biology, and Professor in the Faculty of Health Sciences at McMaster. His work has been published in journals including *Nature*, *Science*, *Nature Medicine*, *Nature Cell Biology*, *Nature Biotechnology*, *PNAS*, *Developmental Cell*, and *Immunity*.

Dr. Bhatia has been honored with; Canada Research Chair in Stem Cell Biology and Regenerative Medicine (Tier 2), Canada's Top 40 Under 40, Krembil Foundation Research Chair in Stem Cell Biology and Regenerative Medicine, Michael G. DeGroot Chair in Stem Cell and Cancer Biology, Canada Research Chair in Human Stem Cell Biology (Tier 1), University of Guelph 2008 Alumni Medal of Achievement, Canadian Society for Biochemistry, Molecular and Cellular Biology (CSBMCB) Scientist Award, and named the 2011 McMaster University Innovator of The Year.

Dr. Bhatia is a recognized leader in human stem cell research. Although stem cells can serve as sources for cellular and organ replacement in tissue damaged by trauma or genetic influences, and for disease intervention, Dr. Bhatia's studies also focus on human cancer and using human stem cells to understand how cancer begins, and how treatment may be revolutionized. Dr. Bhatia's research program seeks to understand the molecular mechanisms that govern somatic and pluripotent human stem cell development through; 1) characterization of molecular pathways and genomic targets that regulate hematopoietic and pluripotent stem cells differentiation, and 2) use of novel *in vivo* models for cellular/tissue regeneration through xeno-transplantation. As a recognized leader in human stem cell biology and applications, Dr. Bhatia's work has been published in several major journals over the years, and his program continues to focus on two central areas; a) developing abundant sources of human hematopoietic progenitors, and b) using human stem cells to develop treatments to eliminate tumor reoccurrence.

Dr. Bhatia also serves as a scientific consultant to government and industry, and to medical companies interested in stem cell-based technologies, and sits on numerous editorial and scientific advisory boards.

John E. Hambor, PhD

Dr. Hambor is a consultant and Director of Stem Cell-based Drug Discovery with the Cell Therapy Group. Dr. Hambor has over 20 years of research, management and training experience in both the pharmaceutical and biotechnology industries. A results-driven

executive leader, Dr. Hambor has a proven ability to apply breakthrough science to the discovery and development of novel small molecules and biological therapies.

As a skilled hands-on scientist, executive and new business architect, Dr. Hambor has core competencies in multiple areas including: Basic Research (stem cell biology, drug discovery, transgenic animals, molecular biology and genetics), Process Development (optimization of stem cell expansion & differentiation into multiple somatic cell types, RNAi & small molecule screening, recombinant protein production); Clinical Manufacturing (SOPs, batch records, automated large-scale cell culture); Project Management (project design & implementation, process reengineering, strategic planning); Supervision (team building, talent development, team & organizational alignment); and External Collaboration (corporate, academic).

Dr. Hambor was formerly the Chief Executive Officer of CellDesign, Inc., a global research and development company that specializes in the development of customizable stem cell tools, primary cells, and reagents for applications in drug discovery & research. Prior to founding CellDesign, Dr. Hambor was the Chief Executive Officer of Cognate BioServices. Cognate is a contract manufacturer of cell-based products providing GMP-quality cells for clinical trials and pre-clinical studies.

Previously, Dr. Hambor was an Associate Research Fellow at Pfizer Global Research and Development. Dr. Hambor joined Pfizer in 1990 in Groton, Connecticut and has worked in several areas of increasing responsibilities. He spent his early years as a cellular and molecular biologist in the Inflammation and Immunology therapeutic areas. In 1998, Dr. Hambor joined the Genetic Technologies Department where he formed a stem cell research unit, eventually overseeing global efforts in stem cell technologies as part of the Genetically Modified Models Center of Emphasis.

Dr. Hambor also holds an adjunct faculty position at Connecticut College where he teaches Immunology. He is author of over 10 patents and 25 peer-reviewed scientific publications, and has been invited to lecture on his work at numerous international conferences. He is an active member of multiple scientific societies, serving as a member of the steering committee for the New York Academy of Sciences, organizing conference programs and chairing panel sessions. He is a scientific consultant for Expedition New England and a member of the Board of Directors for Vivo Biosciences. Dr. Hambor attended Miami University of Ohio where he graduated with B.A. and M.S. degrees in Microbiology. He received a Ph. D. in Pathology from Case Western Reserve University and subsequently moved on to Yale where he did post-doctoral studies in Immunology.

Shoukhrat Mitalipov, PhD

Dr. Mitalipov is Associate Scientist in the Department of Reproductive and Developmental Sciences and Co-Director of the Assisted Reproductive Technologies (ART) and Embryonic Stem Cell (ESC) Core at the Oregon National Primate Research Center. He is also Associate Professor in the Department of Obstetrics and Gynecology, Department of Molecular and Medical Genetics, and the Oregon Stem Cell Center in the School of Medicine at Oregon Health & Science University. He received his PhD in

Developmental and Stem Cell Biology from the Research Center for Medical Genetics at the Russian Academy of Medical Sciences in Moscow, Russia.

The main focus of several ongoing projects in Dr. Mitalipov's lab is to understand the mechanisms of genetic and epigenetic reprogramming of aged somatic cells to the totipotent and pluripotent states following somatic cell nuclear transfer (SCNT). Specifically, he is interested in the role of mitochondria and mitochondrial (mt)DNA in reprogramming and re-setting the developmental program in experimental pluripotent stem cells derived from aged somatic cells.

Dr. Mitalipov's lab is also investigating novel germ line gene therapy approaches for the treatment of inherited human diseases. Particularly, mutations in mtDNA contribute to a diverse range of still incurable human diseases and disorders. mtDNA is maternally inherited through the egg's cytoplasm and it is estimated that at least 1 in 200 born children have an mtDNA mutation that may lead to disease. His team recently demonstrated that in animal models, the mitochondrial genome could be efficiently replaced in mature oocytes by chromosome transfer from one egg to an enucleated, mitochondrial-replete egg. The reconstructed oocytes with the mitochondrial replacement were capable of supporting normal fertilization, embryo development and produced healthy offspring. This discovery suggests that the nuclear genetic material from a patient's egg containing mtDNA mutations could be removed, and transplanted into an enucleated egg containing normal mtDNA donated by a healthy female. A child born following fertilization with the husband's sperm would be free of risk from maternal mtDNA mutations as well as the authentic biological child of the patients. The overall goal of ongoing clinical studies in the Mitalipov lab is to replicate animal studies with human oocytes donated by patients carrying mtDNA mutations after informed consent. Healthy egg donors that commit to donating their oocytes for research will be used as mtDNA donors.

Dr. Mitalipov is an active grant reviewer for the National Institutes of Health (NIH), where he serves on numerous study sections and ad hoc review panels. Additionally he serves on several review boards for other funding agencies including the New York State Stem Cell Research Program, the W. M. Keck Foundation, and the National Science Foundation.

Mark Noble, PhD

Dr. Noble is Professor in the Departments of Genetics, Neurobiology and Anatomy, and Neurology at the University of Rochester Medical Center, Director of the University of Rochester Stem Cell and Regenerative Medicine Institute, and is co-Director of the New York State Center of Research Excellence for Spinal Cord Injury. He received his B.S. degree in Biology and Philosophy from Franklin & Marshall College and his Ph.D. in Genetics from Stanford University.

Dr. Noble was co-discoverer of the first progenitor cell to be isolated from the CNS, the progenitor cell that gives rise to myelin-forming oligodendrocytes. His laboratory then discovered cell-cell interactions and specific mitogens that control the division of these

cells, along with conditions allowing greatly enhanced cell expansion *in vitro*. These discoveries led to the first use of purified precursor cell populations for repair of experimental CNS lesions. His laboratory also discovered adult-specific populations of progenitor cells, and the team of researchers with whom he works has played a central role in the discovery, isolation, and characterization of nearly all of the lineage-restricted progenitor cell populations that have been isolated from the developing CNS, characterization of nearly all of the lineage-restricted progenitor cell populations that have been isolated from the developing CNS, characterized at the clonal levels, and transplanted back into the CNS.

Dr. Noble's current research is focused on developing a comprehensive approach to the field of stem cell medicine, research which includes topics such as identifying the optimal cells for enhancing repair of spinal cord injury; the central importance of precursor cell dysfunction in developmental maladies; and the discovery of molecular mechanisms that underlie effects of environmentally relevant levels of chemically diverse toxicants on CNS precursor cells and that integrate stem cell biology, redox biology, signaling pathway analysis, and toxicology into a mechanistic framework.

Jacqueline Sagen, PhD, MBA

Dr. Sagen is Professor of Neurosurgery at The Miami Project to Cure Paralysis, University of Miami Miller School of Medicine. She has led a cutting-edge research program exploring the use of cell transplantation in restoring function and improving therapeutic options in the injured or diseased nervous system for over 25 years. She received an undergraduate degree in Neuroscience from Northwestern University and her Ph.D. in Pharmacology from the University of Illinois. Due to her interest in translating promising novel cell-based therapies towards clinical implementation, Dr. Sagen also pursued an M.B.A. in Entrepreneurship and held a previous position as Associate Director at CytoTherapeutics, Inc., a biotechnology company targeting cellular transplantation therapies.

Dr. Sagen's current research is focused on neural transplantation and gene therapy strategies to alleviate debilitating consequences following injury to the nervous system, including spinal cord injury, traumatic brain injuries, and peripheral nerve injuries. A particularly disabling consequence of injury to the nervous system is the emergence of chronic pain, which is notoriously difficult to manage using currently available treatments and significantly reduces the quality of life and productivity of afflicted patients. Cellular transplantation can provide a local and continually renewable source of analgesic agents and neurotrophic factors for sustained relief on a long-term or permanent basis, reducing or eliminating the need for repeated pharmacologic treatments and their attendant untoward side effects. Earlier work in Dr. Sagen's lab led to initial clinical trials for cancer pain management using donor-derived adrenal chromaffin cells, with promising outcomes. However, the large-scale feasibility of this approach may be limited by availability of donor tissue, immunological concerns, and scalable cell potency. Thus, more recent work has been directed towards identifying both improved delivery strategies and novel analgesic peptides with distinct targets that can enhance analgesic potency of transplantable cells. In order to accomplish this, embryonic neural stem cells as well as

autologous adult cell sources (e.g. bone marrow stem cells) are being evaluated for delivery of potent analgesic peptides engineered from mammalian and non-mammalian origins. With the emergence of a multitude of potential stem cell sources, and safer and more efficient viral vectors, translatable cell-based therapies for nervous system disorders are now within our reach.

Dr. Sagen has received numerous research grants and awards from the National Institutes of Health (NIH), philanthropic foundations, and private sector biotechnology companies, and has continued to serve on advisory and review panels for NIH, the Department of Defense (DoD), private foundations, University scientific awards programs, and other research funding agencies. She is an active member of the American Society for Neural Therapy and Repair and the International Neural Transplantation and Repair societies. Dr. Sagen also strongly believes in the importance of educating and inspiring the next generation of scientists, and is involved in university-based and community-wide education and training mentorship programs for students ranging from high school through pre-doctoral levels.