Final Agenda

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Cambridge Healthtech Institute's Inaugural...

STEM CELL RESEARCH

A Technology with the Promise to Contribute to All of Medicine

August 29-31, 2005 • University Park @ MIT Hotel • Cambridge, MA

This Conference is running concurrently with Tissue Models for Therapeutics, August 29-30, 2005

KEYNOTES


Dr. Mina J. Bissell, Lawrence Berkeley National Laboratory


Dr. Sheng Ding, Genomics Institute of the Novartis Research Foundation, Scripps Research Institute


Dr. George Q. Daley, Children's Hospital, Boston


Dr. Ihor Lemischka, Department of Molecular Biology, Princeton University

FEATURED SPEAKERS


Dr. Ole Isacson, McLean Hospital/Harvard Medical School, NINDS Morris K. Udall Parkinson's Disease Research Center of Excellence


Dr. Charles A. Vacanti, Harvard Medical School, Brigham and Women's Hospital


Dr. Leonard I. Zon, Children's Hospital, Boston

COMPREHENSIVE COVERAGE ON:

- Stem Cell Research and its Application for Drug Discovery
- Stem Cell Sources, Culture and Expansion
- Stem Cell Research and its Application in Therapeutics

CONFERENCE HIGHLIGHTS:

- Interactive Panel Discussions
- Technology Spotlights
- Exhibit and Poster Viewing
- Networking Opportunities

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Cambridge Healthtech Institute's Inaugural...

STEM CELL RESEARCH

A Technology with the Promise to Contribute to All of Medicine

August 29-31, 2005 • University Park @ MIT Hotel • Cambridge, MA

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Rapid progress in stem cell research has captured the attention of both the public and biotechnology/pharmaceutical sectors and has great potential for the advancement of medical science. As with all new basic scientific and technological knowledge, careful and rigorous examination of the science of stem cell biology is required before unwise applications of stem cell therapy be used. One fundamental question being asked in this field is "what is stemness - how is it maintained and how is differentiation initiated or triggered?" Understanding the complexity of such signaling pathways is critical to advancing the use of these cells for drug discovery and development as well as other therapeutic applications. Cambridge Healthtech Institute's ***Inaugural Stem Cell Research*** addresses two emerging themes in this field: a fundamental understanding of stem cell biology and a view toward drug and therapeutic development.

SUNDAY, AUGUST 28

5:30-6:30 Early Registration

MONDAY, AUGUST 29

7:30 Registration and Morning Coffee

DAY ONE: STEM CELL RESEARCH AND ITS APPLICATION FOR DRUG DISCOVERY

APPLICATIONS IN DRUG DEVELOPMENT AND SCREENING

8:15 **Chair's Opening Remarks**

Dr. Linda Griffith, Director, Biotechnology Process Engineering, Massachusetts Institute of Technology

KICK-OFF KEYNOTE PRESENTATIONS

(combined session with Tissue Models for Therapeutics)

8:30 **Cells and Tissues in Context: Culture Models for the 21st Century!**

Dr. Mina J. Bissell, Distinguished Scientist, Lawrence Berkeley National Laboratory

The extracellular matrix is now widely recognized as an important source of signals for gene expression as well as cell division, survival, shape, and movement, all of which are altered during tumor progression. Construction of more realistic three-dimensional models of normal breast and breast cancer that mimic the normal and diseased conditions would allow gaining new insights into breast tumorigenesis and the findings continue to challenge convention. This presentation highlights the progress of three-dimensional breast cancer models and potential of utilizing this model system to study cancer pathogenesis and test anticancer drugs.

9:15 **Chemical and Functional Genomic Approaches Toward Regenerative Medicine**

Dr. Sheng Ding, Assistant Professor, Departments of Chemistry and Cell Biology, Genomics Institute of the Novartis Research Foundation, Scripps Research Institute

Recent advances in stem cell biology may make possible new approaches for the treatment of a number of diseases. Such approaches could involve cell replacement therapy and/or drug treatment to stimulate the body's own regenerative capabilities, while they will require identification of renewable cell sources of engraftable functional cells, an improved ability to manipulate stem cell proliferation and differentiation, as well as a better understanding of the signaling pathways that control their fate. Cell-based phenotypic and pathway-specific screens of synthetic compounds have recently provided a number of small molecules that can be used to selectively control stem cell fate. Such molecules will likely provide new insights into stem cell biology, and may ultimately contribute to effective medicines for tissue repair and regeneration.

10:00 **Coffee Break**

FEATURED PRESENTATION

10:15 **Use of Zebrafish to Find Stem Cell Genes and Drugs**

Dr. Leonard I. Zon, Grousbeck Professor of Pediatrics, Howard Hughes Medical Institute Investigator, Children's Hospital, Boston

Embryonic stem cells have the ability to make all tissues of the body. Blood cells have been derived from embryonic stem cells using a simple culture media in a semisolid media. Yet, when these cells are transplanted into lethal irradiated mice no reconstitution is evident. In an effort to find factors that increase stem cell numbers and confer a long-term potential to the hematopoietic stem cell, we have utilized the zebrafish system. Zebrafish are amenable to high-throughput screens for chemicals that regulate activity. Several chemical screens are underway to look for stem cell activators. These chemicals are then utilized on embryonic stem cells to evaluate whether there is a potential for long-term reconstitution. A comparative approach to stem cells should provide a better understanding of the pathways necessary for self-renewal and differentiation.

10:45 **Pharmacological Potential of Embryonic Stem Cells**

Dr. Shilpa Kadam, Research Investigator I, Epigenetics Group, Novartis Institutes for BioMedical Research

Stem cells are defined by the ability both to produce identical daughter cells (self-renewal), and to produce progeny with more restricted fates (commitment and differentiation). This property of stem cells underpins growth and diversification during development and sustains homeostasis and repair processes throughout adult life. An understanding of molecular mechanisms which govern stem cell fate is therefore of fundamental significance in cell and developmental biology and the capabilities arising from such knowledge have major biomedical applications. The ease with which these cells can be cultured under *in vitro* conditions serves as an important new tool for developing unique, *in vitro* model systems to test drugs and chemicals, and as a potential to predict toxicity in humans. The current focus of our investigation is to understand the mechanisms of self-renewal and differentiation of stem cells using both genomic and proteomic approaches, and to understand how to manipulate adult stem cells, which hold great promise in regenerative medicine.

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11:15 **Cloning and Stem Cells: Interrogating Development and Disease by Nuclear Transplantation**

Dr. Kevin Eggan, Junior Fellow, The Harvard Society of Fellows, Department of Molecular and Cell Biology, Harvard University

Nuclear transplantation and embryonic stem cell technologies provide a novel route to investigate the mechanisms the underlie both embryonic development and disease progression. Nuclear transplantation using donor cell from individuals affected by genetic diseases would allow the production of embryonic stem cell lines that carry the compliment of genes which cause that disease. By differentiating these "disease specific" embryonic stem cell lines into the affected cells in vitro, the progression and pathogenesis of the disease might bee studied. In addition, studying the nuclear transplantation process itself will provide information into how cells transition from developmental state to another.

11:45 **Panel Discussion with Morning Speakers or Technology Spotlights**

12:15 **Lunch on your own (Technology Workshops Available)**

STEM CELL MODELS FOR SCREENING ASSAYS

(combined session with Tissue Models for Therapeutics)

2:00 **Chair's Remarks**

Dr. Jonathan A. Garlick, Professor and Director of Cancer Biology and Tissue Engineering, Tufts University School of Dental Medicine

2:05 **Stem Cell Models for Target Validation, Drug Screening and Safety Assessment**

Dr. John Hambor, Associate Research Fellow, Experimental Medicinal Chemistry, Pfizer Global Research and Development

Cell models play a significant role in all phases of drug discovery and development from target validation studies, to high-throughput screening of chemical libraries, and finally to testing drug metabolism, transport and toxicity, and they provide critical information allowing selection of safe, efficacious compounds with the best chance of success in the clinic. Recent advances in stem cell technology have led to the development of renewable cultured cell systems that more closely match real human target tissues allowing for more predictive assessment of compound safety and efficacy. Moreover, the advent of simple, quick, robust strategies that reliably lead to differentiation into the desired tissue has permitted the utilization of stem cell derived cell types in screening assays, as well as provided unique opportunities to explore their developmental pathways for the discovery of regenerative small molecules. Progress in applying stem cell models as research tools for target validation, drug screening and safety assessment will be discussed.

2:35 **Using Motor Neurons Derived from Embryonic Stem Cells for Drug Discovery**

Dr. Amy Sinor, Researcher, Assay Development, Curis

We are able to generate cultures derived from embryonic stem (ES) cells that are highly enriched for motor neurons. This ability provides us a unique opportunity to perform high throughput screens in motor neurons. Since these cultures represent the actual cell type that is affected during the progression of many motor neuron diseases, such as Spinal Muscular Atrophy and Amyotrophic Lateral Sclerosis, this provides a strong rationale to use motor neuron cultures to screen for potential drugs. Our pioneer work endorses the great potential of using stem cells for drug discovery. Since ES cells have the ability to differentiate into many different neuronal cell types in cultures, we expect this model system to be valuable for screening drugs for many other neurodegenerative diseases.

3:05 **A Novel Functional Assay of Cell Binding by a Medical Device/Implant**

Dr. Mary Zacour, Principal Scientist, in Vitro Assay Development, Metabolism Resource, MDS Pharma Services

Implanted medical devices or scaffolds that are designed to harness a patient's endogenous stem or precursor cells have enormous potential as a therapeutic approach for a variety of pathologies, since they can target tissue regeneration and/or repair responses to relevant sites in vivo. In order to develop such devices effectively, there is a need for functional in vitro assays, both for assessing the effects of developmental modifications and for eventual quality control of the final product. Many assays of cell binding to scaffolds are largely qualitative, labor-intensive, and very low throughput. We have recently developed a 96-well plate format, quantitative cell-based assay for direct functional assay of cell-binding of a medical device. The assay is adaptable to other devices and cell phenotype specificities, and as such may provide a valuable research tool to developers of such devices.

3:35 **Refreshment Break, Poster and Exhibit Viewing**

ENGINEERING HIGHER THROUGHPUT SCREENING ENVIRONMENTS

4:15 **Using Automated Microcarrier-Based Cell Culture to Improve Human Cell Phenotype**

Dr. Robin Felder, Professor of Pathology and Medical Automation, University of Virginia

3-D cell culturing produces cells that more closely resemble in vivo phenotypes, which will increase their value in screening, research, and ultimately as a cell source for regenerative medicine. Novel microcarriers, bioreactors, and automation systems are being used (and integrated) to allow hands-free growth and maintenance of cells for just-in-time delivery of contamination-free product directly into high throughput screening systems. This talk will focus on and review the technologies available to both the bench scientist and those interested in production-scale cell production.

4:45 **Panel of Experts: Engineering a Functional High-Throughput Tissue Screening System**

High-throughput assay systems (HTS) are needed to most fully take advantage of 3D tissues as a target system for therapeutics. This panel will focus on the issues and challenges facing tissue biologists, pharmacologists and engineers that need to be addressed to accelerate development of these assays. Discussion will include such issues as:

1- Can tissues be scaled to an HTS format?

2- What are the plasticware and media requirements?

3- How can production and screening of 3D tissues be robotized?

Panel members will include experts in each of these areas of HTS application and development. By engineering 3D tissues suitable for HTS, novel screening and therapeutic applications for tissues will be feasible.

Panel Moderator: *Dr. Jonathan A. Garlick, Professor and Director of Cancer Biology and Tissue Engineering, Tufts University School of Medicine*

Panel Participants

Dr. Angela Cacace, Senior Research Investigator II, Lead Discovery, Bristol Myers Squibb Co.

Dr. Linda Griffith, Director, Biotechnology Process Engineering, Massachusetts Institute of Technology

Dr. Robin Felder, Professor of Pathology and Medical Automation, University of Virginia

Dr. John D. McNeish, Senior Director, Genetic Technology, Pfizer Global Research and Development

5:30 **Networking Reception in Exhibit Hall**

6:30 **Close of Day One**

TUESDAY, AUGUST 30

DAY TWO: STEM CELL SOURCES, CULTURE AND EXPANSION

8:30 **Morning Coffee**

8:45 **Chair's Remarks**

KEYNOTE PRESENTATION

8:50 **Stem Cells in Regenerative Medicine and Reproductive Biology**

Dr. George Q. Daley, Associate Professor of Pediatrics, Children's Hospital, Boston

This presentation will highlight the use of embryonic stem cells to unravel the molecular mechanisms of blood and germ cell formation, and the use of somatic cell nuclear transfer to create customized stem cell lines for treatment of genetic disease.

CELL CULTURE

9:35 **Defining the Feeder Activities for Human ES Cell Self-renewal**

Dr. Ren-He Xu, Senior Scientist, Research and Development, WiCell Research Institute

Current culture conditions for human embryonic stem cells (hESCs) require unidentified molecules from mouse or human feeder cells, potentially transmitting xenogeneic or allogeneic pathogens. We demonstrate that hESCs are subjected to high levels of bone morphogenetic protein (BMP) activity when cultured without support of the feeder cells. The BMP

antagonist noggin synergizes with basic fibroblast growth factor to repress BMP signaling and sustain undifferentiated proliferation of hESCs in the absence of feeder cells or feeder-conditioned medium.

10:05 Stem Cell Differentiation using Combinatorial Cell Culture

Dr. Yen Choo, CEO, Plasticell Limited

Plasticell is a biotechnology company that has developed a novel cell culture method 'Combinatorial Cell Culture' - which allows it to assay large numbers (up to millions) of different cell culture protocols in parallel, to derive new methods of growing or differentiating cells. Combinatorial Cell Culture will have widespread utility throughout cell biology, and particularly in the differentiation of stem cells. There the method has been used to study the behavior of pluripotent embryonic stem cells under large numbers of culture conditions, generating new protocols for the directed differentiation of these cells into interesting lineages. Plasticell is focused on identifying and dissecting pathways of cell differentiation with the aim of generating regenerative medicines.

10:35 Coffee Break, Poster and Exhibit Viewing

11:15 New Technologies to Advance Adult Stem Cell Applications

Dr. James L. Sherley, Associate Professor, Div. of Biological Engineering, Massachusetts Institute of Technology

Among the barriers to developing adult stem cell-based applications for biomedicine and biotechnology are the difficulty in expanding them in culture, and the lack of molecular markers that uniquely identify them. We have focused on a unique property of adult stem cells, asymmetric self-renewal, as the basis for developing new technologies for their expansion and identification. Proof of principle has been established in rodent tissue models, and current studies evaluate the application of these technologies to two different human adult stem cell types, liver and blood.

11:45 Interactive Panel Discussion with Morning Speakers

12:15 Lunch on your own (Technology Workshops Available)

STEM CELL SOURCES

1:30 Chair's Remarks

1:35 Technology Spotlights

2:05 Science, Ethics and Therapeutic Potential of Human Stem Cells

Dr. Stephen Minger, Stem Cell Biology Lab, Wolfson Centre for Age-Related Diseases, King's College London

Human embryonic stem cells offer considerable promise in the treatment of significant human disease, but only if the requisite therapeutically important cell populations can be generated. Under license from the UK Human Fertilization and Embryology Authority, our lab has derived three human embryonic stem cell lines, including one that encodes the most common mutation for Cystic Fibrosis. Much of our current work is focused on the clinical translation of human ES cells for CNS, retinal, cardiac, endocrine and hepatic disorders.

2:35 Oct-4-Expressing Cells Isolated from Umbilical Cord Matrix are Multipotential Stem Cells

Dr. Kathy Mitchell, Assistant Professor, Pharmacology and Toxicology, University of Kansas

We have isolated mesenchymal stem cells (MSC) from the human umbilical cord matrix (HUCM). These cells proliferate in culture and express markers found in other stem cells. Specifically, HUCM cells express the transcription factors Oct-4 and nanog, which are important for maintaining the undifferentiated, pluripotent state of embryonic stem (ES) cells. These Oct-4 expressing cells are found in the perivascular region of the umbilical cord. They can be differentiated into multiple cell types including neuronal, endothelial and epithelial cells, and are therefore candidates for cell-based therapies. In contrast to ES cells, but like umbilical cord blood cells, HUCM cells display more immune tolerance and have been shown not to form tumors when injected in immune-compromised mice. Furthermore, these cells are easily accessible compared to bone marrow MSC, are more abundant than the MSC found in cord blood and they lack the ethical considerations of ES cells.

3:05 Hair Follicle Nestin-Expressing Stem Cells can Form Neurons

Dr. Robert M. Hoffman, Professor of Surgery, University of California-San Diego; President, AntiCancer, Inc.

We have recently shown the expression of nestin, the neural-stem-cell marker protein, is expressed in bulge-area stem cells of the hair-follicle. We used transgenic mice with green fluorescent protein expression driven by the nestin-regulatory-element (ND-GFP). The ND-GFP stem cells give rise to the outer-root sheath of the hair follicle as well as to a nestin-expressing interfollicular vascular network. In the present study, we demonstrate that ND-GFP-expressing stem cells isolated from the hair-follicle bulge area that are negative for the keratinocyte marker keratin 15 can differentiate into neurons, glia, keratinocytes, smooth muscle cells, and melanocytes *in vitro*. These pluripotent nestin-GFP-expressing stem cells are positive for the stem cell marker CD34, as well as keratin 15-negative, suggesting their relatively undifferentiated state. The apparent primitive state of the ND-GFP stem cells is compatible with their pluripotency. Furthermore, we show that ND-GFP-expressing stem cell-derived cells can differentiate into neurons after transplantation to the subcutis of nude mice. These results suggest that hair follicle bulge-area ND-GFP stem cells may provide an accessible, autologous source of undifferentiated multipotent stem cells for therapeutic application.

3:35 Refreshment Break, Poster and Exhibit Viewing

LABELING/TRACKING STEM CELLS

4:00 Label-Retaining Epithelial Cells in Mouse Mammary Gland Divide Asymmetrically Retaining Their Template DNA Strands

Dr. Gilbert Smith, Principal Investigator, MBTL,CCR, National Cancer Institute, NIH

An aspect of somatic stem cells that is indicative of their "stemness" is the characteristic of retaining radioactive nucleotides over long periods of time after labeling in vivo. This has been ascribed to their relative mitotic quiescence and/or to their very slow passage through the cell cycle. An alternative view was proposed by John Cairns in 1975. He posited that stem cells dividing asymmetrically in somatic tissues selectively retain their template DNA strands during cell division and partition the newly synthesized strands to their committed (and dispensable) daughters. In this way, somatic stem cells were protected from mutations resulting from errors in DNA replication. As an additional consequence of this, mechanism asymmetrically cycling stem cells would retain radio-nucleotide label received during their inception over long time periods. This characteristic of stem cells would also permit their relative insensitivity to protocols designed to kill cells through the incorporation of nucleoside analogs or senescence due to telomere shortening. Double labeling of long label-retaining mammary epithelial cells in situ provides direct confirmation of the existence of this mechanism for genome protection in the mouse mammary gland. The recent interest in tumor-initiating stem cells and their role in solid tumor maintenance, potentially through asymmetric cell division, marks this aspect of "stemness" as having increasing importance in the design of cancer therapeutics.

4:30 Tracking the Fate of Stem Cells by *in vivo* MRI

Dr. Joseph Frank, Chief, Experimental Neuroimaging Section LDRR, National Institutes of Health

Magnetic labeling of cells provides the ability to monitor their temporal spatial migration in vivo MRI. Various methods have been used to magnetically label cells using coated superparamagnetic iron oxide (SPIO) nanoparticles. In this presentation, I will describe the different approaches used to label cells with contrast agents and show MRI and histologic results in various animal disease models. Magnetic Tagging of stem cells and other mammalian cells has the potential for guiding future cell-based therapies in humans and for the evaluation of cellular-based treatment effects in disease models.

5:00 Interactive Panel Discussion with Afternoon Speakers

5:30 Close of Day Two

WEDNESDAY, AUGUST 31

DAY THREE: STEM CELL RESEARCH AND ITS APPLICATION IN THERAPEUTICS

8:30 Morning Coffee

REALIZING THERAPEUTIC POTENTIAL

8:45 Chair's Remarks

KEYNOTE PRESENTATION

8:50 Stem Cells and Systems Biology

Dr. Ihor Lemischka, Professor, Department of Molecular Biology, Princeton University

Dr. Lemischka's research interests include hematopoietic stem cell biology and developmental biology. The unifying goal of his studies is a direct cellular, molecular and functional analysis of hematopoietic stem cells. Recently his laboratory has been applying genomics and functional genomics approaches to identify stem cell molecular phenotypes and to begin elucidating stem cell regulatory network and pathways.

9:35 **Stem Cell-Based Therapies and the FDA: On the Critical Path to Innovative Medical Products**

Dr. Donald Fink, Jr., Biologist, Office of Cellular, Tissue and Gene Therapies, US Food and Drug Administration

Cellular biologic therapies either consisting of or derived from embryonic, fetal or adult stem cells (stem cell-based therapies) may provide effective treatments for current unmet medical needs that necessitate replacement, restoration, repair, or regeneration of damaged or diseased tissues and organ systems. The challenge of drawing upon all available scientific evidence to assess and gage the safety of biologics produced from stem cells is the responsibility of the Center for Biologics Evaluation and Research (CBER) within the Food and Drug Administration (FDA). The safety and efficacy review of stem cell-based therapies is the responsibility of the Office of Cellular, Tissue, and Gene Therapies (OCTGT), CBER. Regulatory expectations for the development of nascent therapies comprised of stem cells, including those derived from human embryonic stem cells, will be discussed.

10:05 **Manufacturing Cell and Gene Therapy Products - Overcoming the Obstacles**

Dr. Scott Burger, Managing Director, Advanced Cell & Gene Therapy, LLC

Unprecedented numbers of cell and gene therapy products are in clinical development today, for a remarkable range of therapeutic applications. US FDA alone has over 500 INDs cell or gene therapy INDs on file; worldwide more than 300 companies are active in this area. Yet cell and gene therapies present unique challenges in process development, characterization, GMP and GTP manufacturing, and distribution. Overcoming these obstacles requires innovative strategies that blend elements of biotechnology, engineering, transplantation and transfusion medicine.

10:35 **Refreshment Break, Poster and Exhibit Viewing**

11:15 **Transplantation to Regeneration: Industrializing Tissue and Cell Therapies through an Evolving Political and Regulatory Landscape**

Mr. David Smith, Pepper Hamilton, LLP

Clinical applications of human tissue engineering and regeneration technologies are the beneficial end result of an extended process of basic and applied research. However, that research process does not necessarily naturally anticipate and seamlessly resolve the many product development, regulatory approval and market acceptance issues encountered in the transition from bench to bedside. This presentation will provide an overview of those elements – with particular attention to tissue access and regulatory concerns – that can have a significant impact on the economic viability of an emerging tissue and cell therapy industry.

11:45 **Technology Spotlights**

12:15 **Lunch on your own**
(Technology Workshops Available)

THERAPEUTIC APPLICATIONS

1:30 **Chair's Remarks**

FEATURED PRESENTATIONS

1:35 **Advances in Tissue Engineering Related to Stem Cell Biology**

Dr. Charles A. Vacanti, Leroy D. Vandam/Benjamin G. Covino Professor of Anaesthesia, Harvard Medical School; Anesthesiologist-in-Chief & Director, Laboratories for Tissue Engineering and Regenerative Medicine, Brigham and Women's Hospital

A key factor, now receiving much attention, is the source of the cells to be utilized for tissue engineering. Several studies have suggested that immature cells, as opposed to fully differentiated cells of specialized tissues, may hold greater potential for tissue engineering. Such cells may be multipotent (progenitor cells), or pluripotent (stem cells). They can be autologous (adult, mesenchymal stem cells), or from another individual (embryonic stem cells). Immature cells can be induced to differentiate after several divisions. Efforts by several groups have focused on the use of embryonic stem cells. In our laboratories, we have studied a unique adult progenitor/stem cell for Tissue Engineering applications. They appear to lie dormant in several tissues of the body, and are stimulated to proliferate and mature after an insult or injury to the tissue. We have characterized these cells in many tissues including liver, brain, kidney and pancreas and believe that they may be responsible for the natural repair of injured tissue. One area of interest in our labs is in the area of spinal cord regeneration. We have hypothesized that the potential exists to isolate neural stem cells from injured spinal cord and deliver them back into the injured area, in combination with specific polymer scaffolds and various growth factors in a manner to repair the defect. We will present data related to these studies in rats and higher animals.

2:05 **Biological Opportunities and Therapeutic Limitations in the use of Stem Cells for Neurological Disease**

Dr. Ole Isacson, Professor of Neurology (Neuroscience), Harvard Medical School; Director of Center for Neuroregeneration Research at McLean Hospital/Harvard Medical School; and NINDS Morris K. Udall Parkinson's Disease Research Center of Excellence

Clinical studies using transplantation of fetal dopaminergic (DA) cells into the brains of Parkinson's disease patients have provided proof of principle that implanted immature, but well-defined post-mitotic neurons can restore function even in a progressive age-dependent neurological disease. A biotechnological and large-scale medical application of this methodology could be achieved by obtaining similar cells derived from human embryonic stem (hES) cells, or perhaps even by stimulating endogenous adult stem cells. However, while several hES cell differentiation protocols have been developed for generating DA neurons, the production of sufficient amounts of the "right" therapeutic DA cell has not yet been accomplished. To achieve this goal, specific criteria have to be fulfilled to really obtain therapeutically useful DA cells and also a clinical understanding is needed of how to accomplish sufficient cell survival, accurate integration in the brain circuitry with normal function in the absence of tumor formation or immunogenicity in patients. This presentation provides an in-depth discussion of the current state of hES cell-derived neurogenesis and necessary criteria for generating therapeutically relevant cell sources for neurological diseases.

2:35 **Development and Application of Lung Progenitor Cells - State of the Art Technology**

Dr. Min Wu, Assistant Professor, Department of Biochemistry and Molecular Biology, University of North Dakota

The lung possesses a myriad of cell phenotypes because of its unique function of inhaling and expiring air. Due to this structural complexity, transdifferentiation of stem cells into the lung is particularly complicated. In addition, assessing the stem cells and lung progenitor cells in the respiratory system is technically difficult. Despite these difficulties, recent studies have advanced our understanding of bone marrow stem cells differentiating into lung progenitors as well as the local progenitor cells. Our laboratory focuses on mechanistic analysis and therapeutic application of local lung progenitor cells. For analytical purposes we use alveolar epithelial type II cells as a model to define the pathogenic mechanism of P. aeruginosa infection at cellular levels. For therapeutic studies we apply the progenitor type II cells for over-expressing DNA repair genes to reduce oxidative injury. We will be presenting our updated research in lung progenitor development and application. We will also discuss obstacles that limit the use of stem cells in the lung.

3:05 **Use of ESC to Treat Autoimmune Diseases**

Dr. Richard K. Burt, Chief, Division of Immunotherapy, Northwestern University Feinberg School of Medicine

ESCs, (embryonic stem cells) have been the topic of much professional and lay literature, yet despite their potential, the use of ESCs to cure disease is generally lacking. Herein, we demonstrate that ESCs may be easily manipulated without adverse side effects to treat and cure type I diabetes through induction of islet cell tolerance.

3:35 **Development of Unrestricted Somatic Stem Cells (USSCs) from Umbilical Cord Blood for Cardiac Regeneration after Myocardial Infarction.**

Dr. Stephan Wnendt, Senior Vice President, R&D, ViaCell Inc.

Interventional cardiology and cardiac surgery have brought major therapeutic achievements to the treatment of coronary artery disease and myocardial infarction by catheter-mediated revascularization and bypass-grafting. However, even after successful restoration of coronary blood flow after acute myocardial infarction many patients suffer from chronic remodeling of the left ventricle, leading to impaired cardiac function and reduced life expectancy. Several clinical trials with catheter-based or surgical transplantation of autologous bone marrow-derived cells or skeletal myoblasts indicate that cell therapy can improve cardiac function by prevention or even reversion of left ventricular remodeling. In order to provide a pluripotent cell source of standardized quality as an off-the-shelf product we are developing our human cord-blood derived USSC for cardiac cell therapy. Pre-clinical data from small and large animal models of myocardial infarction show improvement of relevant parameters of cardiac function (e.g. ejection fraction, wall motility, stroke work) and engraftment of human cells after USSC transplantation. The presentation will provide an overview on the current cardiac cell therapy and the development of USSC as an allogeneic stem cell source for cardiac regeneration.

4:05 **Interactive Panel Discussion with Afternoon Speakers**

4:30 **Close of Conference**

Stem Cell Research Foundation

A program of the American Cell Therapy Research Foundation

Stem Cell Research Foundation:

It is the mission of the Stem Cell Research Foundation to help find treatments and cures for a wide range of diseases by supporting innovative research into the development of cell therapies.