STEM CELLS:
BIOLOGY AND APPLICATIONS

26th Annual International Symposium

Friday, May 31, 2013
8:30am-6:00pm

Hunter College of the City University of New York
695 Park Avenue
Hunter West Building, Room 714
(*East 68th Street at Lexington Avenue*)
New York, NY 10065

Supported by the National Institute on Minority Health and Health Disparities,National Institutes of Health (Grant #8 G12 MD007599-27) and the Weill Cornell Medical College Clinical and Translational Science Center (Grant #2UL1TR000457-06) of the National Center for Advancing Translational Sciences of the National Institutes of Health.

SYMPOSIUM PROGRAM

http://genecenter.hunter.cuny.edu/symposia/stem-cells

26th Annual International Symposium

Sponsors:
Hunter College of the City University of New York,
Center for Study of Gene Structure and Function

Weill Cornell Medical College,
Clinical and Translational Science Center

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Memorial Sloan-Kettering Cancer Center, Hospital for Special Surgery,
Hunter College School of Nursing, Cornell University Cooperative Extension

The 26th Annual International Symposium of the Center for Study of Gene Structure & Function at Hunter College, with Weill Cornell Medical College Clinical and Translational Science Center, is supported by the National Institute on Minority Health and Health Disparities, National Institutes of Health (Grant #8 G12 MD007599-27) and the National Center for Advancing Translational Sciences (Grant #2UL1TR000457-06)

The Research Centers in Minority Institutions (RCMI) Program was transferred to the National Institute on Minority Health and Health Disparities (NIMHD) following the passing of the Consolidated Appropriations Act, 2012, which dissolved the National Center for Research Resources, the former home of the RCMI program.

The RCMI Program of the National Institutes of Health develops and strengthens the research infrastructure of minority institutions by expanding human and physical resources for conducting basic, clinical, and translational research. It provides grants to institutions that award doctoral degrees in the health professions or health-related sciences and have a significant enrollment of students from racial and ethnic minority groups that are underrepresented in biomedical sciences. The program also serves the dual purpose of bringing more racial and ethnic minority scientists into mainstream research and promoting minority health research because many of the investigators at RCMI institutions study diseases that disproportionately affect minority populations.

NIMHD

The mission of the National Institute on Minority Health and Health Disparities (NIMHD) is to promote minority health and to lead, coordinate, support, and assess the NIH effort to reduce and ultimately eliminate health disparities. In this effort NIMHD will conduct and support basic, clinical, social, and behavioral research, promote research infrastructure and training, foster emerging programs, disseminate information, and reach out to minority and other health disparity communities. http://www.nimhd.nih.gov/default.html

NATIONAL INSTITUTES OF HEALTH

The NIH, a part of the U.S. Department of Health and Human Services, is the primary federal agency for conducting and supporting medical research. Composed of 27 Institutes and Centers, the NIH provides leadership and financial support to researchers in every state and throughout the world. Its mission is science in pursuit of fundamental knowledge about the nature and behavior of living systems and the application of that knowledge to extend healthy life and reduce the burdens of illness and disability. http://www.nih.gov

RTRN
RESEARCH CENTERS IN MINORITY INSTITUTIONS TRANSLATIONAL RESEARCH NETWORK

RTRN is strategically positioned to facilitate interdisciplinary clinical and translational research. RTRN has established a solid technological foundation to support intellectual exchange, generate innovative inter- and multi-disciplinary research and facilitate the movement of scientific advances throughout the translational research spectrum.

Center for Study of Gene Structure and Function

The Center for Study of Gene Structure and Function (Gene Center) at Hunter College of the City University of New York was established in 1985 through the vision of James Wyche, Harvey Ozer (former Program Coordinators for the Gene Center) and Richard Mawe (former Program Director of the Gene Center) with the support of the Research Centers in Minority Institutions (RCMI) Program of the now defunct National Center for Research Resources of the National Institutes of Health (NIH). In 2012, the RCMI Program was transferred to the National Institute on Minority Health and Health Disparities (NIMHD) of the National Institutes of Health (NIH).

The Gene Center is a consortium of researchers from the Hunter College departments of biology, chemistry, psychology, physics, anthropology, and urban public health. Since the Gene Center's inception, the growing number of papers published in peer-reviewed journals and the number and amount of grants obtained by the faculty have been the most visible hallmarks of its success. The Gene Center provides a vibrant research environment marked by workshops on cutting-edge research techniques; frequent research colloquia and seminars by guest scientists; an annual international symposium, which is a major event on the New York scientific calendar; and a strong emphasis on collaborative translational research. In addition, the Gene Center encourages bright undergraduates, especially minorities, to develop a career in biomedical research by hosting a Summer Program for Undergraduate Research and supports the professional development of minority scientists through the JustGarciaHill science web site.

The Gene Center is a key partner in the Clinical and Translational Science Center, an enterprise that also includes the Weill Cornell Medical College, Memorial Sloan-Kettering Cancer Center, the Hospital for Special Surgery and the Hunter College School of Nursing. The Clinical Translational Science Center was established in 2007 with the aim of accelerating translational research. The overall goal is to facilitate the transition of laboratory work into state-of-the-art clinical research (T1 research), provide research that improves patient care (T2) and health outcomes in the general community (T3). The Gene Center also participates in a national consortium, the Research Centers in Minority Institutions Translational Research Network (RTRN that facilitates collaboration, large-scale projects, and sharing of facilities among Research Centers in Minority Institutions.

The Gene Center is supported by the Research Centers in Minority Institutions Program of the National Institute on Minority Health and Health Disparities of the NIH. Grant # 8 G12 MD007599-27.

Visit the Gene Center website: http://genecenter.hunter.cuny.edu

Clinical and Translational Science Center

The Clinical and Translational Science Center (CTSC) is a unique collaboration between renowned biomedical and community organizations centered on Manhattan's east side. Weill Cornell Medical College and the Graduate School of Medical Sciences is home to the administrative core of the CTSC, led by CTSC Program Director Julianne Imperato-McGinley, MD, Associate Dean of Translational Research and Education at Weill Cornell Medical College (WCMC).

In addition to WCMC, the CTSC partner institutions include:

- Hunter College, Center for Study of Gene Structure and Function
- Hunter College, School of Nursing
- Hospital for Special Surgery
- Memorial Sloan-Kettering Cancer Center
- Cornell University Co-operative Extension in New York City

Affiliated hospitals include New York-Presbyterian Hospital, Lincoln Medical Center, Methodist Hospital, New York Downtown Hospital, New York Queens Hospital, Wyckoff Heights Medical Center, and Brooklyn Hospital.

The CTSC is designed to bring together the resources of all partner and affiliate institutions to facilitate novel translational research. Separately, these institutions include superb academic centers of excellence, a diverse patient base, and a unique community-engagement program designed to foster collaboration between community groups and translational research scientists. Each partner and affiliate has an unmistakable character that enhances multi-disciplinary interaction. Integration of these unique resources and intellectual assets will facilitate translation of research findings in the laboratory to clinical research at the bedside and ultimately to best practices within underserved communities.

This center is funded through the Clinical and Translational Science Awards (CTSAs), a national consortium that is transforming how clinical and translational research is conducted.

For more information about the CTSC, please visit http://www.med.cornell.edu/ctsc.

STEM CELLS: BIOLOGY AND APPLICATIONS

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The potential of stems cells and induced pluripotent stem cells (IPSCs) to be differentiated and returned to one's own body as a therapeutic source of "replacement" cells or tissue is on the horizon. The symposium will describe the basic science of IPSC and other stem cell developments, pioneering experiments and ethical implications.

The morning session will focus on the ability to derive pluripotent stem cells and their ability to be differentiated into specific populations for therapeutic purposes. **James Thomson** *(Director of Regenerative Biology, Morgridge Institute for Research at University of Wisconsin)* is a pioneer in the production of human IPSCs and will discuss avenues of generating such cells. **Christine Mummery** *(Professor of Developmental Biology and Chair of the Department of Anatomy and Embryology, Leiden University Medical Center, the Netherlands)* and **Lorenz Studer** *(Director, Center for Stem Cell Biology at Memorial Sloan-Kettering Cancer Center)* will discuss directed differentiation into specific subtypes. In particular, Dr. Studer will discuss recent clinical trials involving these cells. **Paul Feinstein** *(Associate Professor, Hunter College of the City University of New York)* will discuss ongoing work to improve the quality and speed of IPSC generation.

The first afternoon session will delve into the applied science aspects of directed differentiation and the ethical implications of obtaining biological material and reintroducing modified versions back into patients. **Harriet Washington** *(Award Winning Writer and Editor)* has authored several books on medicine and society and will focus on disparities in participation, risks and benefits surrounding the use of ES cells. **Koen van Besien (**Professor of Medicine, Weill Cornell Medical College**)** will discuss placental origins of stem cells as well as the difficulty in obtaining these cells from the population at large. **Paula Cannon** *(Associate Professor, Molecular Microbiology & Immunology, Pediatrics, Biochemistry & Molecular Biology, Keck School of Medicine, University of Southern California)* and **Benjamin Ortiz** *(Associate Professor of Biology, Hunter College of the City University of New York)* will describe the usage of ES cells in the treatment of HIV and development of immunological lineages, respectively.

The second afternoon session will start by discussing the mechanism of tissue regeneration in an invertebrate with its potential implications for cell replacement in mammals by reinvigorating inherent stem cell populations. The symposium will conclude by discussing tissue engineering or synthesis in the lab with the possibility of reintroduction into the body. **Alejandro Sanchez Alvarado** *(Professor of Neurobiology and Anatomy, University of Utah)* will discuss the stem cells abundant in planarians and its implications on regeneration biology. **Peter Zandstra** *(Canada Research Chair in Stem Cell Bioengineering, Institute of Biomaterials and Biomedical Engineering, The Donnelly Centre for Cellular and Biomolecular Research, University of Toronto)* and **Gordana Vunjak-Novakovic** *(Professor and Vice-Chair, Department of Biomedical Engineering, Director, Laboratory for Stem Cells and Tissue Engineering, Columbia University)* will discuss efforts to bring differentiated cells back to the body as tissues or organs.

MORNING SESSION: Pluripotency and Directed Differentiation

9:00	Introduction and welcome: **Jesus Angulo,** Professor of Biological Sciences at Hunter College, CUNY and Program Director of the Center for Study of Gene Structure and Function **Jennifer J. Raab,** President, Hunter College, CUNY **Julianne Imperato-McGinley,** Associate Dean of Translational Research, Weill Cornell Medical College
9:15	**James Thomson, Keynote Speaker** Director of Regenerative Biology, Morgridge Institute for Research, University of Wisconsin *Title: Human Pluripotent Stem Cells*
9:50	**Christine Mummery** Professor of Developmental Biology and Chair of the Department of Anatomy and Embryology, Leiden University Medical Center, the Netherlands *Directed Cardiac Differentiation of Pluripotent Stem Cells and Potential Applications*
10:15	**Lorenz Studer** Director, Center for Stem Cell Biology, Memorial Sloan-Kettering Cancer Center *Human ES Cell Derived Midbrain Dopamine Neurons for Cell Therapy in Parkinson's Disease*
10:40	**Paul Feinstein** Associate Professor of Biological Sciences, Hunter College of the City University of New York *Generation of Induced Pluripotent Stem Cells by Cell Fusion*
11:00	**Panel Discussion: Questions & Answers Session**
11:30	Lunch for **pre-registered** participants, Poster Session

AFTERNOON SESSION 1: Applications and Implications

1:00	**Remarks by Sponsoring Agency**
1:20	**Harriet Washington, Keynote Speaker** Award-winning Medical Writer & Editor *Stem Cell Research: Disparate Participation, Risks, and Benefits*
1:55	**Koen van Besien** Professor of Medicine, Weill Cornell Medical College *Hematopoietic Stem Cell Transplantation: The Search for the Perfect Donor*
2:20	**Paula Cannon** Associate Professor, Molecular Microbiology & Immunology, Pediatrics, Biochemistry & Molecular Biology, Keck School of Medicine, University of Southern California *Using Stem Cells to Build an HIV-Resistant Immune System*
2:45	**Benjamin Ortiz** Associate Professor of Biological Sciences, Hunter College of the City University of New York *Locus Control Region Activity in T Cells Derived in Vitro From Embryonic Stem Cells*
3:10	**Panel Discussion: Questions & Answers Session**
3:40	**Coffee Break, Poster Session and Book Signing by Harriet Washington**

AFTERNOON SESSION 2: Tissue Engineering and Regeneration

4:00 **Alejandro Sánchez Alvarado, Keynote Speaker**
Professor of Neurobiology and Anatomy, University of Utah
The Developmental Biology of Regeneration

4:35 **Peter Zandstra**
Canada Research Chair in Stem Cell Bioengineering, Institute of Biomaterials and Biomedical Engineering, The Donnelly Centre for Cellular and Biomolecular Research, University of Toronto
Two Short Examples of Engineering Cell Fate:Blood Stem Cell Therapy and Cardiac Drug Screening

5:00 **Gordana Vunjak-Novakovic**
Professor and Vice-Chair, Department of Biomedical Engineering, Director, Laboratory for Stem Cells and Tissue Engineering, Columbia University
Engineering Human Tissues

5:25 **Panel Discussion with Questions & Answers Session**

5:55 **Poster Awards Ceremony and Raffle**

6:00 **Concluding Remarks**

Presentations will be available via the symposium web site.

James Thomson, Ph.D.

Morgridge Institute for Research,
University of Wisconsin

Human Pluripotent Stem Cells

Abstract: Whether derived from the early embryo (embryonic stem cells) or from reprogrammed adult cells (induced pluripotent stem cells), human pluripotent stem cells share two basic properties: they divide without limit, and they can form any cell of the body. These properties give researchers unprecedented access to the basic building blocks of the human body, with implications for basic biology, drug development, and transplantation medicine. Dr. Thomson will discuss the basic properties of human pluripotent stem cells, their implications for human medicine, and the challenges that lie ahead.

Bio: Thomson graduated with a B.S. in biophysics from the University of Illinois in 1981. He entered the Veterinary Medical Scientist Training Program at the University of Pennsylvania, receiving his doctorate in veterinary medicine in 1985, and his doctorate in molecular biology in 1988. He derived the first human embryonic stem (ES) cell line in 1998 and derived human induced pluripotent stem (iPS) cells in 2007.

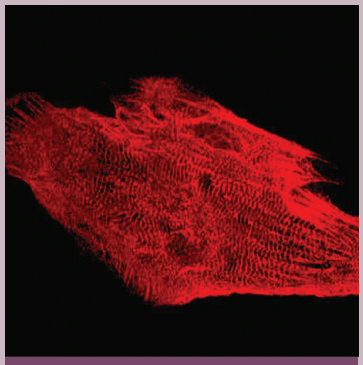
He serves as Director of Regenerative Biology at the Morgridge Institute for Research in Madison, Wisconsin, is a professor in the Department of Cell and Regenerative Biology at the University of Wisconsin's School of Medicine and Public Health and a professor in the Molecular, Cellular, and Developmental Biology Department at the University of California, Santa Barbara. He is also a founder and Chief Scientific Officer for Cellular Dynamics International, a Madison-based company producing derivatives of human induced pluripotent stem cells for drug discovery and toxicity testing.

Christine Mummery, Ph.D.

Leiden University Medical Center, the Netherlands

Directed Cardiac Differentiation of Pluripotent Stem Cells and Potential Applications

Abstract: Derivation of heart cells from human pluripotent stem cells (hPSC) is an area of growing interest as a platform for drug discovery and toxicity. Understanding the underlying developmental mechanisms controlling differentiation to cardiac progenitors and mimicking these in defined culture conditions in vitro is now essential for moving the field forward. Culture conditions have now been sufficiently refined that cardiomyocyte is an efficient and reproducible process. Genetically marked hPSC have been produced by targeting in which expression of GFP is driven by specific lineage markers. Here, we describe hESC and hiPSC lines in which GFP has been targeted to the cardiac progenitor locus Nkx2.5. We have used this tagged line to optimize differentiation protocols, derive isogenic disease and control lines and to select cardiomyocytes to examine their responses to different cardiac and non-cardiac drugs. The results showed high correspondence with readouts derived from the model systems used by pharma in cardiac safety pharmacology, indicating their potential value in cardiac lead compound discovery. Additional studies showed that combinations of drugs or specific genetic backgrounds modelled using cardiomyocytes from patient derived hiPS cells predispose to increased sensitivity to drugs affecting potassium channels. We also show that it is possible to model a complex cardiac "overlap syndrome" caused by a specific mutation in a cardiac sodium channel that recapitulates the phenotype seen in both adult mice and the patient. Together, this type of approach to safety pharmacology demonstrates the utility of human PSCs in modelling disease and providing new platforms for drug discovery.



cardiomyocyte

Bio: Christine is Professor of Developmental Biology at Leiden University Medical Centre, The Netherlands and she is chair of the Department of Anatomy and Embryology. Her research has largely concerned mouse cardiovascular development and the role of growth factors. Over the last 10 years this has extended to the directed differentiation of mouse and human embryonic stem cells to cardiomyocytes and vascular cells. She pioneered studies characterizing cardiomyocytes from hES cells and was among the first to inject them in mouse heart and assess their effect on myocardial infarction. Her more recent work has concerned creating cardiac and vascular disease models based on induced pluripotent stem cells and their potential use in drug safety pharmacology and drug discovery.

She has recently written a popular book on stem cells "Stem Cells: scientific facts and Fiction" (2011) intended as a semi-lay guide to stem cell biology and applications. She is also the founding editor in chief of Stem Cell Reports, the new journal of the ISSCR, editorial board member of Cell Stem Cells and Stem Cells and past president of the International Society of Differentiation (2010-2012). In 2010 she was elected as a member of the Royal Netherlands Academy of Arts and Science. In the same year she became a member of the board of the academy. She was recently awarded an ERC Advanced grant to continue research on cardiovascular derivatives of human pluripotent stem cells and their use as disease models.

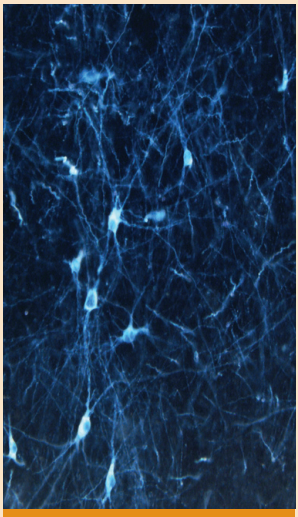
Lorenz Studer, M.D.

Memorial Sloan-Kettering Cancer Center

Human ES Cell Derived Midbrain Dopamine Neurons for Cell Therapy in Parkinson's Disease

Abstract: Parkinson's disease (PD) is characterized by the loss of midbrain dopamine neurons that is responsible for the characteristic motor symptoms of the disease. Dopamine neuron replacement has been proposed as an experimental therapeutic strategy since the 1980s with more than 300 patients grafted world-wide using human fetal tissue. While those studies have demonstrated the feasibility of dopamine neuron replacement the clinical results have been mixed.

One main limitation of fetal tissue grafting has been the limited supply and the variable nature of tissue for transplantation. Over the last 5 years there has been renewed interest in the dopamine neuron replacement paradigm, triggered by a re-analysis of grafted patients > 10 years after transplantation suggesting remarkable long-term benefit in a subset of individuals. Furthermore, novel sources of dopamine neurons have become available to resolve issues such as limited supply or variability of the tissue for transplantation. Here we will review our data on the use of human ESC-derived dopamine neurons in mouse, rat and monkey models of PD. Directed differentiation protocols yield large numbers of authentic dopamine neurons via a human ESC floor plate intermediate. Furthermore, those dopamine neurons show excellent in vivo survival and function in the various PD models. Finally, we will discuss remaining challenges for translating pre-clincial findings towards clinical use and present strategies to further improve the function and safety of human ESC derived DA neurons; efforts geared towards the first clinical trial of human ESC-derived dopamine neurons in PD patients.



Human ESC Derived Dopamine Neurons after Transplantation into Monkey Brain

Bio: A native of Switzerland, Lorenz Studer graduated from medical school in 1991 and received his doctoral degree in neuroscience at the University of Bern in 1994. While there, he initiated studies with Christian Spenger, leading to the first clinical trial of fetal tissue transplantation for Parkinson's disease in Switzerland. Studer next pursued his research interests at the National Institutes of Health (NIH) in Bethesda, Maryland, where he worked in the laboratory of Ron McKay. At the NIH he pioneered the derivation of dopamine cells from dividing precursor cells. In 1998, he was first to demonstrate that the transplantation of dopamine cells generated in culture improve behavioral symptoms in Parkinsonian rats.

In 2000, he moved to New York City where he started his research program at the Memorial Sloan-Kettering Cancer Center (MSKCC). Early contributions of his lab include the in vitro derivation of midbrain dopamine neurons from ES, nuclear transfer ES cells and parthenogenetic stem cells. His laboratory was also first to demonstrate "therapeutic cloning" in a mouse model of a CNS disorder, and he has pioneered studies on the directed differentiation, high-throughput screening and genetic modification of human ES cells. His most recent work increasingly focuses on the translational application of human pluripotent stem cells in disease modeling, drug discovery and cell therapy. He currently leads a large multidisciplinary consortium to pursue the first clinical application of human ES cell derived dopamine neurons for the treatment of Parkinson's disease. He received numerous awards for his work including the Boyer Young Investigator award and, most recently, the Annemarie Opprecht Award.

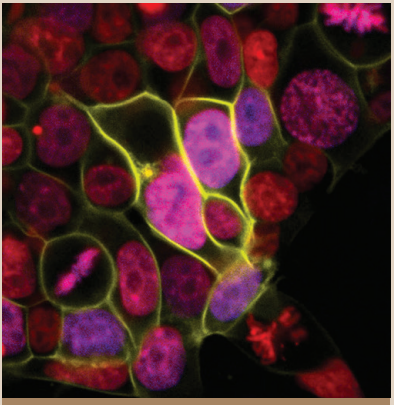
Studer is the Director of the Sloan-Kettering Center for Stem Cell Biology. He is a Member of the Developmental Biology Program and the Department of Neurosurgery at MSKCC and a Professor in Neuroscience at Weill-Cornell.

Paul Feinstein, Ph.D.

Hunter College of the City University of New York

Generation of Induced Pluripotent Stem Cells by Cell Fusion

Abstract: The generation of induced pluripotent stem cells (IPSCs) and their subsequent differentiation into defined lineages represents a major step forward in the biomedical field, having significant clinical, disease-modeling and drug-testing applications. Diploid IPSCs were first produced by reprogramming mouse somatic cells with the Yamanka factors and current methods are still based on variations/enhancements thereof. However, the time frame for production of IPSCs remains long, especially for human IPSC generation. Alternatively, reprogramming somatic cells by fusion with embryonic stem cells (ESCs) takes only a few days, but the resultant cells contain tetraploid nuclei and thus are not useful for therapeutic purposes. My laboratory is investigating whether reprogrammed diploid nuclei can be generated by fusion of ESCs with somatic cells. As a complementary goal we are investigating high-throughput approaches for the identification of the correct karyotype of ESC and IPSC lines.



An embryonic stem cell colony with nuclei marked in red and the plasma membrane marked in yellow.

A subset of the cells are observed in the Metaphase stage of mitosis.

Bio: Paul Feinstein, Ph.D. is Associate Professor of Biology at Hunter College. A graduate of the University of Pennsylvania in 1986, he obtained his Ph.D. in 1996 at Columbia University in molecular genetics and did his postdoctoral work at The Rockefeller University. Research in the Feinstein laboratory is focused on the generation of induced pluripotent stem cells (IPSCs), genetic manipulation, and mechanisms of olfactory function. For the last two decades, he and his colleagues have created hundreds of gene-targeted and transgenic mice for the study of how odorant receptors function in odor identification, neuronal maturation, axonal path finding, axonal identity and as a regulator of gene choice. During this time, he became an expert in the growth and manipulation of embryonic stem cells (ESCs). Dr. Feinstein was co-author on the first cloning of a mouse from the postmitotic nucleus of a (olfactory) neuron by nuclear transfer (nt) into oocytes, followed by generation of ntESCs and, subsequently, the production of a live animal with ntESCs injected into tetraploid chimeras. His interest in generation of IPSCs is derived from an experiment published by another group whereby ESCs fused with somatic cells lead to rapid reprogramming of somatic nuclei and the generation of tetraploid ESCs.

Harriet Washington

Award-winning Medical Writer & Editor

Stem Cell Research: Disparate Participation, Risks, and Benefits

Bio: Harriet Washington is an award-winning medical writer and editor, and the author of the best-selling book, Medical Apartheid: The Dark History of Medical Experimentation on Black Americans from Colonial Times to the Present. In her work, she focuses mainly upon bioethics, history of medicine, African American health issues and the intersection of medicine, ethics and culture.

Medical Apartheid, the first social history of medical research with African Americans, was chosen as one of Publishers' Weekly Best Books of 2006. The book also won the National Book Critics Circle Nonfiction Award, a PEN award, 2007 Gustavus Myers Award, and Nonfiction Award of the Black Caucus of the American Library Association. It has been praised in periodicals from the Washington Post and Newsweek to Psychiatric Services, the Economist, Social History of Medicine and the Times of London and it has been excerpted in the New York Academy of Sciences' Update. Experts have praised its scholarship, accuracy and insights.

Washington wrote Medical Apartheid while she was a Research Fellow in Ethics at Harvard Medical School. She has worked as a Page One editor for USA Today, as a science editor for metropolitan dailies and several national magazines, and her award-winning medical writing. Her work has appeared in Health, Emerge and Psychology Today, as well as such academic publications as the Harvard Public Health Review, the Harvard AIDS Review, Nature, The Journal of the American Medical Association, The American Journal of Public Health and the New England Journal of Medicine. Her awards include the Congressional Black Caucus Beacon of Light Award, two awards from the National Association of Black Journalists and a Unity Award from Emerge. She is the founding Editor of The Harvard Journal of Minority Public Health and has presented her work at universities in the U.S. and abroad.

In her most recent book, Deadly Monopolies: The Shocking Corporate Takeover of Life Itself, Washington takes an in depth, eye-opening look at the 40,000+ patents on human genes and their harmful, even lethal, consequences on public health. Her other books include, Parkinson's Disease, a monograph published by Harvard Health Publications, Living Healthy with Hepatitis C and she is co-author of Health and Healing for African Americans. Ms. Washington has taught at venues that include New School University, SUNY, the Rochester Institute of Technology, University of Rochester, Harvard School of Public Health and Tuskegee University. She has sat on the boards of many organizations, including The Young Women's Christian Association, the School Health Advisory Board of the Monroe County Department of Health and the Journal of the National Medical Association, to name a few.

Ms. Washington has also worked as a laboratory technician, as a medical social worker, as the manager of a poison-control center/suicide hotline, and has performed as an oboist and as a classical-music announcer for WXXI-FM, a PBS affiliate in Rochester, N.Y. She lives in New York City with her husband Ron DeBose.

Koen van Besien , M.D.

Weill Cornell Medical College

Hematopoietic Stem Cell Transplantation: The Search for the Perfect Donor

Abstract: Hematopoietic stem cell or bone marrow transplant constitutes by far the most common clinical application of stem cells. Hematopoietic stem cells are residing in the adult bone marrow from where they ensure blood production. They can be mobilized into the blood of adults, and are naturally occurring in the blood of newborns. Transplantation of adult bone marrow or peripheral blood stem cells or transplantation of cord blood stem cells is widely used for treatment of patients with leukemia or other disorders of blood production. Since the initial experiments in the late 1950's tremendous advances have occurred and over one million transplants have been performed worldwide. But many challenges and opportunities for improvement also remain. We will briefly discuss the methods and recent advances in this field.

Bio: Koen van Besien serves as the Director of the Stem Cell Transplant program at Weill Cornell Medical Center and New York Presbyterian Hospital. He received his undergraduate degree from the Facultés Universitaires at Namur and his medical degree from the University of Leuven, both in Belgium. After postgraduate training in Belgium and at Indiana University, he became a member of the transplant program at MD Anderson Cancer Center in Houston. He was the director of the transplant and lymphoma programs at the University of Chicago from 2001 until 2011.

Dr. van Besien's research interests include bone marrow transplantation and lymphoma treatments. He has most recently focused on the development of novel transplant conditioning regimens and on improving umbilical cord blood transplantation. He is the author or coauthor of over 200 publications. He is editor in Chief of Leukemia and Lymphoma, a member of the editorial boards of Biology of Blood and Marrow Transplantation and Bone Marrow Transplantation, and a frequent reviewer for other journals. He is an active clinician and is board certified in Internal Medicine, Hematology and Oncology

Paula Cannon, Ph.D.

University of Southern California
Keck School of Medicine

Using Stem Cells to Build an HIV-resistant Immune System

Abstract: The absence of functional CCR5 in individuals homozygous for the CCR5delta32 mutation is associated with profound resistance to HIV, and transplantation of hematopoietic stem cells (HSC) from a CCR5delta32 homozygous donor into a single HIV+ leukemia patient led to the first reported HIV cure in humans. Together, these data suggest that modification of a patient's own HSC to create CCR5-negative cells could be used to build an HIV-resistant immune system in people with HIV/AIDS. Using targeted nucleases such as zinc-finger nucleases (ZFNs) and TALENs, we are able to efficiently knock-out the CCR5 gene in human HSC, and our pre-clinical studies using humanized mice confirmed that such CCR5-negative HSC are able to generate mature CD4+ T cells that resist HIV infection in vivo. Translating this to the clinic requires scale-up and adaptation of the technology, as well as selection of an appropriate patient population for first-in-man clinical trials. In addition, targeted nuclease engineering of HSC is providing a way to achieve gene repair, and thereby providing new approaches for genetic diseases of the blood and immune system that are currently treated by donor HSC transplantation.

Bio: Paula Cannon PhD is an Associate Professor of Microbiology at the Keck School of Medicine at the University of Southern California, where she leads a research team that studies viruses, stem cells and gene therapy. She obtained her PhD from the University of Liverpool in the United Kingdom, and received post-doctoral training as an HIV scientist at both Oxford and Harvard Universities. Although HIV remains the main focus of her work, she also studies highly pathogenic hemorrhagic fever viruses, including Ebola and Lassa fever viruses. Dr. Cannon has a long-standing interest in the development of gene therapy as a clinical approach to treating HIV infection and her recent work in this area is aimed at disrupting the viral co-receptor, CCR5, using zinc finger nucleases (ZFNs). This approach is being evaluated in human hematopoietic stem cells, to address whether such a therapy could result in a 'functional cure' for AIDS patients. Dr. Cannon's research is funded by both the National Institutes of Health and the California Institute for Regenerative Medicine.

Benjamin Ortiz, Ph.D.

Hunter College of the City University of New York

Locus Control Region Activity In T Cells Derived In Vitro From Embryonic Stem Cells

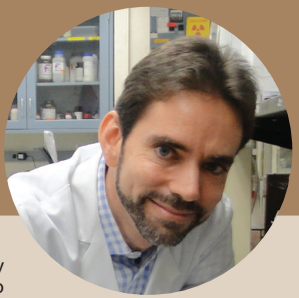
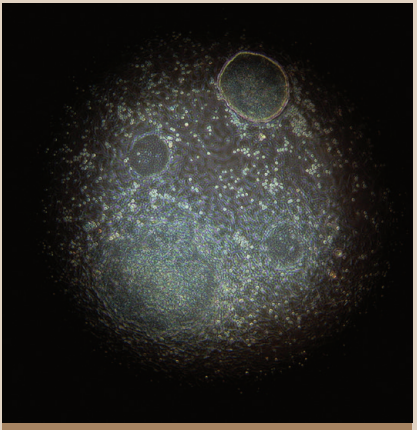


Photo credit to Judith O'Brien

Abstract: Locus Control Regions (LCR) are cis-acting gene regulatory elements with the unique, integration site-independent ability to transfer the characteristics of their locus-of-origin's gene expression pattern to a linked transgene in mice. LCR activities have been discovered in numerous T cell lineage expressed gene loci. These elements can be adapted to the design of stem cell gene therapy vectors that direct robust therapeutic gene expression to the T cell progeny of engineered stem cells. Currently, transgenic mice provide the only experimental approach that wholly supports all the critical aspects of LCR activity. In this talk I will describe our discovery that all key features of mouse T cell receptor (TCR)-α gene LCR function can be manifested in T cells derived in vitro from mouse embryonic stem cells (ESC). High level, copy number-related TCRα LCR-linked reporter gene expression levels are cell type-restricted in this system, and upregulated during the expected stage transition of T cell development. We also confirmed that de novo introduction of TCRα LCR linked transgenes into existing T cell lines yields incomplete LCR activity. Together, these data indicate that establishing full TCRα LCR activity requires critical molecular events occurring prior to final T-lineage determination. This study additionally validates a novel, tractable and more rapid approach for the study of LCR activity in T cells, and its translation to therapeutic genetic engineering. [This work was initiated under NYS-DOH-NYSTEM grant C024302 (B.D.O.) and completed with the support of National Institutes of Health grants SC1-GM095402 (to B.D.O.) and RR003037/MD007599 (to Hunter College)].



"Embryonic stem cells (large, bright, round colonies, top right) first differentiate into mesoderm (dull, flattened colony, bottom left) and then to blood cell types (small bright cells) in vitro."
Photo credit Armin Lahiji

Bio: Benjamin Ortiz, Ph.D. is Associate Professor of Biology at Hunter College. He pursues research on gene regulation during T cell development, while fostering the professional development of numerous members of Hunter's diverse and talented student body. His laboratory has trained a diverse array of Hunter College alumni who are now pursuing biomedical research careers across the country. His lab studies a Locus Control Region (LCR), a DNA segment harboring potent gene regulatory activity in the T cells of the immune system. His lab has most recently pioneered the study of LCR activity in T cells derived in vitro from embryonic stem cells. This breakthrough promises to speed the translation of basic research on LCR activity to the design of gene therapy strategies against diseases such as cancer, inherited immunodeficiencies and AIDS.

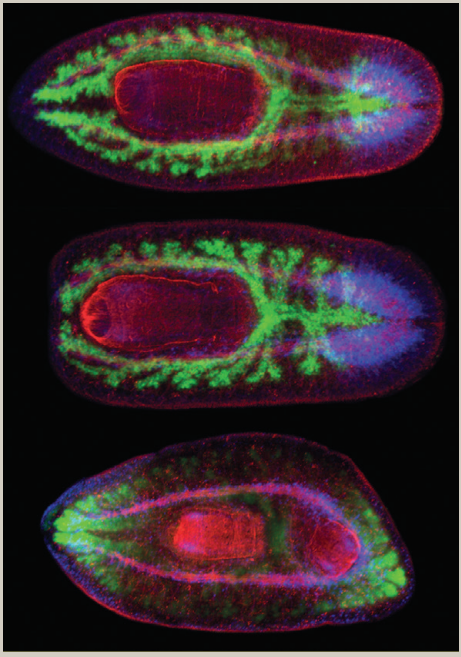
Dr. Ortiz is a Brooklyn native and product of the NYC public schools. He received his B.A. in Biology at Hunter College. The Minority Access to Research Careers (MARC) program at Hunter supported his first research experience, obtained in the laboratory of Dr. Robert Dottin. He was awarded a Howard Hughes Medical Institute Predoctoral Fellowship, with which he went on to earn his Ph.D. in Immunology from Stanford University working in the laboratory of Dr. Alan Krensky. He then conducted postdoctoral research at the University of California, Berkeley in the laboratory of Dr. Astar Winoto. Dr. Ortiz has been on the Hunter College Faculty since 2000. His research has been awarded several grants including a National Science Foundation (NSF) CAREER award, and SCORE and "R01" grants from the National Institutes of Health (NIH). He was also among the first recipients of individual investigator research grants from the Empire State Stem Cell Research Program (NYSTEM). He has served on grant review panels for the Department of Defense, NSF and NIH.

Dr. Alejandro Sánchez Alvarado, Ph.D.

University of Utah

The Developmental Biology of Regeneration

Abstract: "Research into the *in vivo* activation and migration of multipotent progenitor cells will improve our understanding of tissue homeostasis and organ regeneration in multicellular organisms. Due to their general inaccessibility, however, the study of stem cells in vivo continues to be a challenge. In an attempt to overcome this limitation, we have chosen to study the abundant and experimentally accessible stem cells (neoblasts) of the flatworm *Schmidtea mediterranea*. Neoblasts are the only mitotically active somatic cells in planarians and as such are responsible for replacing tissues lost to turnover and/or injury (amputation). Here, I will discuss our efforts to characterize the functions, behaviors and development of these cells, as well as the post-mitotic tissues in which they reside."



Bio: Alejandro Sánchez Alvarado is an Investigator of the Stowers Institute for Medical Research and the Howard Hughes Medical Institute. He received his Bachelor's Degree in Molecular Biology and Chemistry from Vanderbilt University in 1986, and his Ph.D. in 1992 in Pharmacology and Cell Biophysics at the University of Cincinnati School of Medicine, where he studied mouse embryonic stem cells and their *in vitro* differentiation under the tutelage of Dr. Jeffrey Robbins and Dr. Thomas Doetschman. In 1994, he joined the laboratory of Dr. Donald D. Brown at the Carnegie Institution of Washington, Department of Embryology as a postdoctoral fellow, and in 1995 was appointed Staff Associate. It was during this period that Dr. Sánchez Alvarado began to explore systems in which to molecularly dissect the problem of regeneration. In 2002, he became an Associate Professor and was promoted to full Professor in 2004 in the Department of Neurobiology and Anatomy at the University of Utah School of Medicine, where he was later appointed H.A. & Edna Benning Presidential Professor (2010). Dr. Sánchez Alvarado has served in the National Advisory Council, National Institutes of General Medical Sciences, NIH (2008-2012), and is currently on the scientific advisory boards of the Mount Desert Island Biological Laboratory, Bar Harbor, MA; The Eugene Bell Center for Regenerative Biology and Tissue Engineering, Woods Hole, MA; the UCL Centre for Stem Cells and Regenerative Medicine, London, UK; the Institute for Stem Cell Biology and Regenerative Medicine, Bangalore, India; and the Latin American Society for Developmental Biology. He is also a Kavli Fellow of the National Academy of Sciences USA, and the recipient of a MERIT award from the National Institutes of Health and the E.E. Just Medal for Scientific Achievement, from the American Society for Cell Biology. He is also co-director of the Embryology course at the Marine Biological Laboratory in Woods Hole, MA. Dr. Sánchez Alvarado's current research efforts are aimed at elucidating the molecular and cellular basis of animal regeneration using the free-living flatworm *Schmidtea mediterranea.*

Peter Zandstra, Ph.D.

Centre for the Commercialization
of Regenerative Medicine, University of Toronto

*Two Short Examples of Engineering Cell Fate:
Blood Stem Cell Therapy
and Cardiac Drug Screening*

Abstract: Spatial organization and intercellular (between cell) communication networks are an important components of the stem cell microenvironment. These higher order interactions maintain homeostasis and coordinate regenerative and developmental cues in multicellular organisms. We have developed a number of new tools to measure and control cell-cell interactions. These include: high throughput systems for screening paracrine interactions between cells; network analysis strategies (and toolboxes) to depict and analyze ligand connectivity between stem cells, their progeny and cells in their microenvironment; and artificial stem cell niches to regulate and control the context and impact of these interactions. In this presentation I will review the key design principles of these tools, and discuss their application in two short examples. In the first example I will describe our recent work in controlling feedback signaling from differentiated cells to grow human blood stem cells in a clinical relevant bioprocess. In the second example I will present our efforts to formulate human pluripotent derived cardiac cells into micro-tissues that allow for high-throughput functional analysis of responses to drug candidates.

Bio: Research in the Zandstra Laboratory is focused on the generation of functional tissue from adult and pluripotent stem cells. His groups' quantitative, bioengineering-based approach strives to gain new insight into the fundamental mechanisms that control stem cell fate and to develop robust technologies for the use of stem cells and their derivatives to treat disease. Specific areas of research focus include blood stem cell expansion and the generation of cardiac tissue and endoderm progenitors from pluripotent stem cells. Dr. Zandstra is a Professor in the Institute of Biomaterials and Biomedical Engineering, the Department of Chemical Engineering and Applied Chemistry, and the Donnelly Centre at the University of Toronto. He is also a member of the McEwen Centre for Regenerative Medicine and the Heart and Stroke/Richard Lewar Centre of Excellence. He currently acts as Chief Scientific Officer for the Centre for the Commercialization of Regenerative Medicine (www.CCRM.ca). Dr Zandstra's accomplishments have been recognized by a number of awards and accolades including a Guggenheim Fellowship and the McLean Award. Dr Zandstra's strong commitment to training the next generation of researchers is evidenced by his role as the Director of the undergraduate Bioengineering Program.

Gordana Vunjak-Novakovic Ph.D.

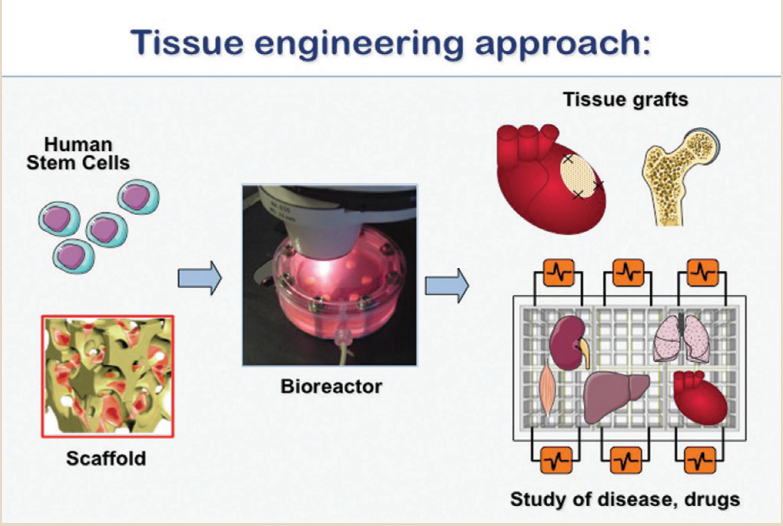
Columbia University

Engineering Human Tissues

Abstract: The overall objective of all tissue engineering is to fully restore the lost tissue function. Engineered tissues of sufficiently high fidelity can also provide physiologically relevant yet controllable models for fundamental research - for example, to study stem cells in a native-like three-dimensional context of development or disease. The utility of tissue engineering depends on our ability to predictably direct the cells to express the right phenotype in the right place and at the right time.

The focus of our research is on engineering functional human tissues, by an integrated use of stem cells ("tissue engineers"), biomaterial scaffolds (cell-instructive templates) and bioreactors (culture environments designed to regulate tissue development). This talk will discuss the overall approach to tissue engineering and some of recent advances in engineering of fully viable and functional human tissues for transplantation, study of disease and drug screening. One translational study in which facial reconstruction was achieved using engineered bone will be presented in more detail as a case study.

Bio: Gordana Vunjak-Novakovic is the Mikati Foundation Professor of Biomedical Engineering, and a Professor of Medical Sciences at Columbia University in New York. She directs the Laboratory for Stem Cells and Tissue Engineering, the Stem Cell Imaging Core, the Bioreactor Core of the national Tissue Engineering Center, and the Stem Cell Core at Columbia University. She is the lead for bioengineering for the Columbia Stem Cell Initiative, and serving on the Board of Directors of the Center for Advancement of Science in Space. The focus of her research is on engineering functional human tissues using stem cells, biomaterials and bioreactors, for regenerative medicine and study of development and disease. She is extensively published and cited (320 papers, 12,000 citations), has 62 patents, and gave 260 invited lectures. Gordana is a frequent advisor to government and industry, a distinguished editor for NIH, a member of editorial boards of 16 scientific journals, and a member of the National Academy of Engineering of the United States.



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