StemCONN 2021

Regenerative Medicine in the 21st century

StemCONN Virtual Conference

April 1 & April 8, 2021

Consecutive Thursday Mornings

9:00 a.m. – 12:30 p.m.

Connecticut's Stem Cell and
Regenerative Medicine Symposium

StemCONN 2021
Regenerative Medicine in the 21st century

Dear StemCONN Participant,

On behalf of the organizing committee, welcome to StemCONN 2021. We are thrilled to host our 7th StemCONN conference, where we celebrate 16 years of achievement in stem cell and regenerative medicine research in Connecticut. StemCONN 2021 not only highlights our cutting-edge stem cell research advancements and provides a forum for discussion among scientists and policy makers, it also at this very unique time, showcases research efforts using stem cells to study responses to viral infections like SARS-CoV2, the cause of the COVID19 pandemic. Because attending on-line conferences allows flexibility with scheduling, StemCONN 2021 will be held on the mornings of April 1 and April 8th allowing participants time to process what they've learned and meet with the speakers in the afternoons. (Note that most of the live content will also be available to registrants as recordings for 1 year following the conference on our website: StemCONN.org.)

We bring together leading investigators from around the world to highlight the very best in stem cell research including basic science discoveries, bioscience industry collaborations, and advances in translational research that have truly enhanced patient care. We feature partnerships between academic researchers and the Connecticut bioscience industry that are advancing knowledge, stimulating new collaborations, and fostering biomedical developments in the state and region.

It is a very exciting time in stem cell research, as new discoveries in the laboratory lead to medical breakthroughs and clinical therapies never before imagined. The world-class science that will be shared at StemCONN 2021 embraces these themes. StemCONN 2021 marks the 16th anniversary of the ground-breaking legislation that has propelled Connecticut to the forefront of stem cell and regenerative medicine research. The state's investment in bioscience initiatives continues to pay dividends for its citizens — supporting major research advances, making possible state-of-the-art medical and research facilities, and creating commercial successes and jobs.

StemCONN 2021 demonstrates the success of bioscience as a vehicle for medical progress and economic growth by emphasizing the power of academia-industry partnerships and faculty entrepreneurship through biotech startups as a means to effectively move scientific discovery to clinical applications. Science education is a critical part of StemCONN's mission. We welcome scientific trainees including high school students, college students, graduate students, postdoctoral fellows and research associates. Registrants will have a special opportunity to meet with most of our invited speakers through afternoon "Meet the Expert" sessions. Two trainees will be honored with the Milton B. Wallack Trainee Award for Excellence in Stem Cell and Regenerative Medicine Research. We thank all of our conference sponsors for their generous financial support. Please look through the sponsor list for the names of these contributors, and during the breaks, and please be sure to access our Sponsors' content on our website StemCONN.org.

Thanks also to my colleagues on the StemCONN 2021 organizing committee for their enduring commitment and contributions to planning this conference. Have a great conference — and remember the essence of science is asking questions and seeking answers — so ask a question at StemCONN 2021 today!

Diane Krause, M.D., Ph.D.

Chair
StemCONN 2021 Organizing Committee

UCONN | UNIVERSITY OF CONNECTICUT

On behalf of the University of Connecticut, I am pleased to welcome you to StemCONN 2021: Regenerative Medicine in the 21st Century. This is a wonderful opportunity for scientists, policy makers, industry partners, and students to advance knowledge and forge new collaborations in this critical area of path-breaking research.

The University of Connecticut is a proud contributor to the state's efforts to shape and guide the stem cell and regenerative medicine fields. Since 2006, the UCONN Stem Cell Core has made major contributions to Connecticut's stem cell initiatives by making available a central source of technologies and research materials to scientists in academia and industry. A collaborative effort that draws on UCONN Storrs, UCONN Health, and the Jackson Laboratory for Genomic Medicine in Farmington, the Stem Cell Core represents the best of what can be accomplished through initiatives like Bioscience Connecticut, which to date has resulted in nearly two dozen biotech startup companies.

This fall, UCONN will also welcome the first cohort of students to pursue the newly created Master's of Science in Regenerative Engineering, the first program of its kind in the world. Training a transdisciplinary workforce in regenerative engineering – which sits at the convergence of stem cell science, developmental biology, materials science, and other disciplines — will enable Connecticut to be at the forefront of developments in this field, like the limb regeneration research being conducted at UCONN.

The University of Connecticut is committed to this work, and to the partnerships with industry leaders, government agencies, and other world-class universities that make it possible. Across efforts like Venture Development and Technology Incubator Program (TIP), we places a high premium on working with innovators and entrepreneurs in these fields. To date, TIP has generated more than 700 patents, with roughly $463 million raised by its companies in the most recent fiscal year.

As we build on the early fruits of these collaborations, the University is helping chart a course for bioscience in Connecticut that will make the state an international center of advancement and industry; indeed, it isn't too much to say these efforts will be "regenerative" for our state in more than one way.

Sincerely,

Thomas Katsouleas

President
University of Connecticut

WESLEYAN
UNIVERSITY

On behalf of Wesleyan University, welcome to StemCONN 2021. Wesleyan is honored to partner with so many important contributors to Connecticut's stem cell and regenerative medicine research ecosystem in support of this year's conference.

In keeping with this year's theme of Regenerative Medicine in the 21st Century, I take special pride in noting Dr. Alison O'Neil's lecture on the biochemistry of neurodegenerative diseases (based on her research with human-induced pluripotent stem cell-derived motor neurons derived from patient populations). In her role as assistant professor, Dr. O'Neil closely collaborates with faculty in and outside of the Chemistry department, as well as both undergraduate and graduate students, serving as a model for how we conduct scientific research at Wesleyan.

Dr. O'Neil and all our life sciences faculty's work in this area exemplifies our university's deep, long-standing commitment to interdisciplinary study. Their research also undergirds Wesleyan's scholar-teacher model, in which we take discoveries made in the laboratory and bring them directly into the classroom. Funding from the state of Connecticut has been crucial in these efforts- enabling faculty and students' eager pursuit of stem cell research in our productive and multifaceted program; helping train new generations of researchers with on-campus workshops; and supporting the off campus impact of this research in scientific publications and outreach that helps educate the public on stem cell therapies for diseases.

Real-world impact is always at the heart of our work at Wesleyan, and StemCONN 2021 is a model for how public and private institutions can work together to achieve it. I am delighted for all Wesleyan will learn from these productive dialogues and collaborations, and for what we can offer to advance our partners' understanding and efforts.

Sincerely,

Michael S. Roth

President
Wesleyan University

Yale

Welcome to the seventh StemCONN Conference! Over the course of this event, you will have opportunities to learn about exciting scientific developments from leading experts and entrepreneurs from Yale and other academic institutions across Connecticut, throughout the country, and around the world.

Connecticut's support of the biosciences and stem cell research attracts renowned investigators and helps us build advanced, versatile facilities. Our community of scientists, policy makers, and bioscience industry partners move the field forward and contribute to the fiscal health of the state. All your efforts bring in research funding, create new jobs, establish companies, and retain our highly skilled workforce.

I am proud that the Yale Stem Cell Center is part of this rich and active community. By working together, you deepen our understanding of stem cell biology and regenerative medicine and harness its potential to improve human health. On behalf of Yale University, thank you for participating in this important endeavor.

Sincerely,

Peter Salovey

President and Chris Argyris Professor of Psychology
Yale University

THE JACKSON LABORATORY
LEADING THE SEARCH FOR TOMORROW'S CURES

It is with great pleasure that The Jackson Laboratory (JAX) for Genomic Medicine joins Connecticut's leading academic institutions to co-sponsor StemCONN 2021. JAX's entry into the Connecticut research community may have been relatively recent, but we are already making great strides in life science research and advancing genomic medicine. Our stem cell research is a central component of our overall research program, and we are exploring both the basic biology and clinical applications of stem cell development, programming and maintenance.

JAX is excited to participate in and contribute to StemCONN and the outstanding Connecticut stem cell and regenerative medicine research effort. We are also proud to be part of the Connecticut state stem cell core grant and collaborate with our neighbors and peers in this vital research area. The potential for stem-cell based medical advances has never been greater, and Connecticut is well-positioned to be a leader in translating genomic research progress to the clinic.

Sincerely,

Charles Lee, Ph.D., FACMG

Director
The Jackson Laboratory for Genomic Medicine

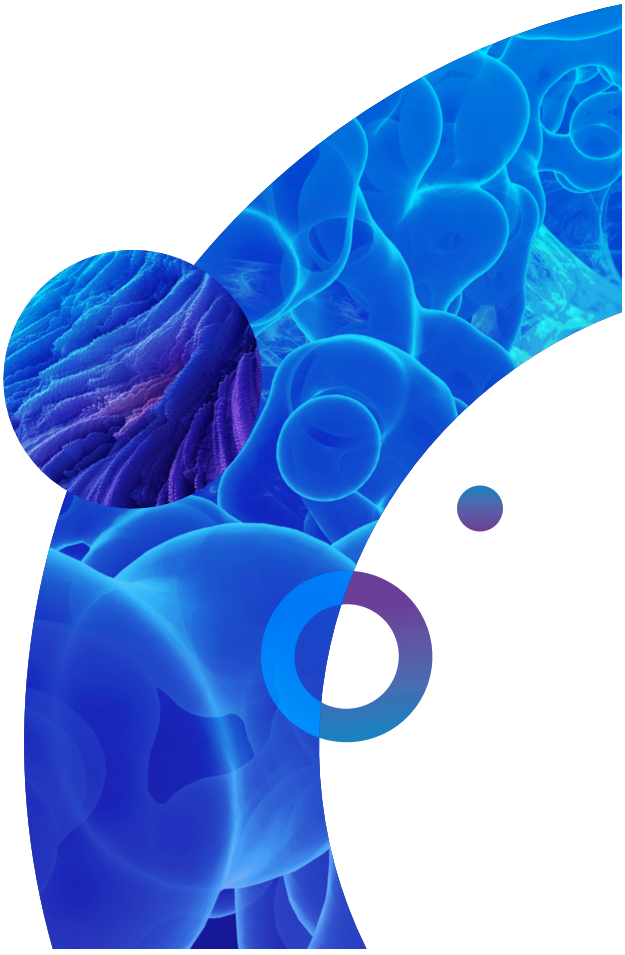
10x GENOMICS

Explore biology like never before

We are in a time of transition. It's not just the age of genomics, it's the century of biology. Scientists on the cutting edge will discover how single cells work in concert through time and space, adding new dimensions to our understanding of biology and human health. Join the exploration.

What will be your discovery?

Chromium Single Cell Solutions
Visium Spatial Solutions



Connecticut Stem Cell – Regenerative Medicine Research Update – 2021

The State of Connecticut continues to be an acknowledged leader in the field of stem cell/regenerative medicine research. The prestigious journal "Cell Stem Cell" in 2015 cited CT together with California as having developed one of the two leading state-supported stem cell programs in the nation. This is an enormous achievement and it reflects the unique collaborative relationship that has been established among Yale, University of Connecticut, Wesleyan and Jackson Laboratory (JAX).

The genesis of our state supported program dates back to 2005 when only one other such state program existed. This was made possible, when CT lawmakers passed legislation that included a commitment of 100 million dollars in state funding for stem cell research over a 10 year period. This support has been leveraged to attract more than 750 million dollars of federal (NIH) and philanthropic funding. As a result, we now have more than 650 scientists across the state engaged in this important stem cell work. Currently, there are more than 250 projects in process, ranging from basic to translational research as well as clinical application with the prospects for commercialization of new therapies and continued significant job growth. We have therefore become a leading "hub" in this field and it is why continued state support is so opportune and important.

In addition, we have seen the formation of a number of companies in CT related to stem cell/ regenerative medicine research. We have also seen the establishment at Yale of a GMP clinical cell processing facility which allows stem cells to be used for clinical trials in stem cell therapy. At UCONN, a single cell analysis facility has been launched jointly with JAX. We are now seeing the development of cell-based treatments for Parkinson's disease, diabetes, blood related disorders, macular degeneration, Alzheimer's disease, epilepsy, Angelman and Prader-Willi syndromes, hypoparathyroidism, stroke, spinal cord injuries, cancer, arthritis, ALS, cartilage and bone repair, and skin related problems, as well as vascular and cardiac diseases.

Over the past year, many CT scientists pivoted their expertise to address the critical challenges of COVID-19. Yale Stem Cell Center (YSCC) researchers have developed approaches to hopefully prevent COVID-19, assays to detect the disease, and drugs to treat the disease. At the YSCC, 12 laboratories and more than 20 of its scientists are currently engaged in COVID-19 related research. At Wesleyan, JAX and UCONN as well, multiple investigators are applying their expertise to treating COVID19. Amongst these CT institutions, areas of investigation include the development of novel therapeutic candidates such as neutralizing antibodies, pilot development of novel coronavirus vaccine candidates, analysis of the differences in immune responses in children vs. adults with COVID-19, research to demonstrate that pharmacological inhibition of platelet aggregation may improve survival in patients hospitalized with COVID-19, efforts to treat moderate and severe COVID-19 patients with a new drug that blocks the assembly and proliferation of the coronavirus, exploration of herbal medicines for treating COVID patients, and work to establish a portfolio of options to mitigate new variants of coronavirus now and in the future.

Aside from this important COVID-19 work, CT's stem cell scientists have continued to make steady progress in stem cell and regenerative medicine research. This includes, among other projects, gene editing utilizing CRISPR technology, further utilization of induced pluripotent stem cells and greater utilization of stem cells for drug testing. Together, JAX, Wesleyan, UCONN and Yale are continuing their efforts to advance "Personalized-Precision Medicine." All of these efforts contribute to further establishing CT as a "hub" for biomedical research and for the further development of medical breakthroughs and the creation of many more new jobs in Connecticut.

Dr. Milton Wallack

Founder, Connecticut Stem Cell Coalition

Milton B. Wallack Trainee Award for Excellence in Stem Cell Research

StemCONN 2021 will recognize excellence in research conducted by predoctoral or postdoctoral trainees through a merit-based award that recognizes highly innovative and important stem cell research. This year 2 awards will be bestowed. The Milton B. Wallack Trainee Award for Excellence in Stem Cell Research is presented in honor of Dr. Milton B. Wallack, a founder of the Connecticut Stem Cell Coalition, a longtime member of the Connecticut Stem Cell Research Advisory Committee, and a consultant to the Regenerative Medicine Research Fund Advisory Committee. The naming of this award both acknowledges and honors Dr. Wallack for his passionate support for stem cell research in the state of Connecticut. Through his efforts to bring together diverse interests toward a common purpose, Connecticut passed the Stem Cell Investment Act in 2005 — an Act supported by our state legislators, former Governor Rell, and the people of Connecticut that committed $100 million in stem cell research funding over ten years. Dr. Wallack then worked with others to create the Regenerative Medicine Research Fund under Governor Malloy, which continued funding for stem cell and regenerative medicine research to the present.

Thanks in large part to these initiatives, Connecticut is now one of the top contributors to stem cell research. Success in this groundbreaking research support required countless hours to initiate the Connecticut Stem Cell Coalition, lobbying and establishing meaningful relationships with members of the state legislature, including former State Senator (now federal senator) Chris Murphy, forging long-lasting connections between investigators throughout the state, and working with Connecticut Innovations. Since passage of the bill, Dr. Wallack's tireless efforts continue in maintaining relationships with people in the legislature, assuring that Connecticut stem cell funding has continued to this day.

Dr. Wallack has played a critical role in organizing each biennial StemCONN meeting, and served for many years as a devoted and hardworking member of the Stem Cell Research Advisory Committee (now called the Regenerative Medicine Research Advisory Committee), written countless editorials to local newspapers and business publications in favor of federal and state stem cell funding, helped Yale and UCONN with fundraising efforts, and has served as a member of the Connecticut Economic Development Committee.

Dr. Wallack promotes collaborative scientific research between universities in Connecticut and Israel, as well as raises funds to support these international collaborations. Dr. Milton Wallack has served the Connecticut stem cell community with unflagging optimism, diligence, and hard work. Dr. Wallack truly lives a life devoted to making the world a better place. In addition to all that he has done for Connecticut's stem cell endeavors, he has contributed significantly to the Jewish community, juvenile diabetes care and research, and educational efforts throughout the state and the country. His leadership is worthy not only of honor but also emulation.

2021 Milton B. Wallack Trainee Award Recipient

Ann Chen, BS

Yale University

Paired Histopathological and scRNA-seq Analysis Reveals that a Subset of Cancer Stem Cells Contributes to Glioblastoma Invasion

Glioblastoma (GBM) is the most aggressive primary brain cancer and remains incurable. In this study, we profiled GBM invasion through cross-platform analysis of human histology, PDX model and scRNA-seq data from ten GBM patients. We report that while cancer stem cells (CSCs) correlate with GBM invasion, only a subset of CSCs contributes significantly to invasive phenotype. Taken together, this work elucidates CSC heterogeneity within GBM and identifies new molecular targets to regulate GBM invasion.

Ann Chen is a fifth-year Ph.D. candidate in biomedical engineering at Yale in the laboratory of Professor Jiangbing Zhou. Ms. Chen's research is focused on using bioinformatics, CRISPR, and nanoparticles to design cancer stem cell-targeting therapies that improve glioblastoma response to current treatments.

2021 Milton B. Wallack Trainee Award Recipient

Dea Gorka, BS

UCONN

Silencing of Paternal UBE3A by UBE3A-ATS Occurs through Transcriptional Interference

This talk focuses on gaining a better understanding of how the interaction between UBE3A-ATS and UBE3A leads to imprinting of UBE3A on the paternal allele in chromosome 15q11-13 in order to learn more about Angelman syndrome (AS) and discover novel therapeutic options for these patients. AS is a neurodevelopmental disorder characterized by motor dysfunction, intellectual disability, severe seizures, absent speech, and a happy demeanor. AS is caused by loss of function from the maternally inherited allele of UBE3A. In most cell types, UBE3A is expressed from both the maternal and paternal alleles. In mature neurons, UBE3A is only expressed from the maternally-inherited allele. Thus, loss of function from the maternal allele leads to nearly complete loss of UBE3A RNA and protein. Imprinted (maternal-only) expression of UBE3A occurs because the paternal allele of UBE3A is silenced by a long non-coding antisense RNA, termed UBE3A-ATS. Activation of paternal UBE3A through the suppression of UBE3A-ATS transcription is a promising therapeutic strategy for AS, however the exact mechanism underlying UBE3A imprinting by UBE3A-ATS is not fully understood. This research uses patient-derived human iPSCs and their neural derivatives to carefully dissect the mechanisms by which UBE3A-ATS silences paternal UBE3A and how antisense oligonucleotides (ASOs) unsilence paternal UBE3A.

Dea Gorka is currently a 4th year Ph.D. student in the Genetics and Developmental Biology (GDB) area of concentration within the Biomedical Sciences Ph.D. program at the University of Connecticut School of Medicine. Her research focuses on using patient-specific induced pluripotent stem cells (iPSCs) to better understand genetic and cellular deficits underlying two 15q imprinting disorders: Angelman syndrome (AS) and Prader-Willi syndrome (PWS).

Times proximate and subject to change.

Thursday, April 1, 2021

9:00 a.m. **Welcome Remarks & Introductions**

Diane Krause, M.D., Ph.D. (StemCONN Committee Chair)
Yale University

In-Hyun Park, Ph.D.
Yale University

Generation of Integrated Brain Organoids to Investigate Brain Development and Diseases

Alison O'Neil, Ph.D.
Wesleyan University

Modeling ALS with Human Stem Cell Derived Motor Neurons

9:50 a.m. **Break**

Se-Jin Lee, M.D., Ph.D.
UCONN, The Jackson Laboratory for Genomic Medicine

Myostatin: A Molecular Rheostat for Muscle Mass

Milton B. Wallack Trainee Award Recipient
Ann Chen, BS

Paired Histopathological and scRNA-seq Analysis Reveals that a Subset of Cancer Stem Cells Contributes to Glioblastoma Invasion

10:45 a.m. **Break**

Welcome Back

Doug Melton, Ph.D.
Harvard University

Using Stem Cells to Make Designer Islets for Diabetics

Samira Musah, Ph.D.
Duke University

Molecular and Biophysical Control of Stem Cell Fate

12:20 p.m. **Closing Remarks**

Haifan Lin, Ph.D.
Yale University, Vice President of ISSCR

12:30 p.m. **Adjourn until April 8th**

"Meet the Expert" Sessions

Several speakers will hold their own sessions at the conclusion of the day.

Must be pre-registered. Visit StemCONN.org to register.

Please note that there will be a moderated Q&A session after each speaker.

Thursday, April 8, 2021

9:00 a.m. **Welcome Back Remarks**

Angela Cacace, Ph.D.
Arvinas

Application of Human Stem Cell Models for Drug Discovery In Monogenic Neurologic Diseases'

Karolina Palucka, M.D., Ph.D.
The Jackson Laboratory for Genomic Medicine

Human-Derived Ex Vivo Systems for Studies on Lung Immunity to Respiratory Viruses

2021 Milton B. Wallack Trainee Award Recipient
Dea Gorka, BS

Silencing of Paternal UBE3A by UBE3A-ATS Occurs through Transcriptional Interference

Michael Bronson, Ph.D.
10x Genomics

Explore Biology Like Never Before with 10x Genomics

10:10 a.m. **Break**

Welcome Back

Allen Eaves, OBC, M.D., Ph.D., FRCPC
StemCell Technologies, Inc.

Starting a Biotech – Going Over to The Dark Side or Seeing the Light

Connie Eaves, Ph.D., FRS (Canada) FRS (Edinburgh)
Terry Fox Laboratory

Normal and Malignant Stem Cells – Is Conceptual Simplicity Going Beyond Utility?

12:20 a.m. **Closing Remarks**

Diane Krause, M.D., Ph.D.
Yale University

12:30 p.m. **Adjourn**

"Meet the Expert" Sessions

Several speakers will hold their own sessions at the conclusion of the day.

Must be pre-registered. Visit StemCONN.org to register.

Angela Cacace, Ph.D.

Vice President of Neuroscience and Platform Biology, Arvinas

Application of Human Stem Cell Models for Drug Discovery in Monogenic Neurologic Diseases'

Recent advances in induced pluripotent stem cells and primary myoblasts have enabled modeling of human neurologic diseases with patient derived cell models. Utilization of iPSC-derived neurons and myoblasts enables screening and evaluation of drug efficacy as well as development of patient stratification and efficacy biomarkers. These cells have the genetic background of patients that more precisely model disease-specific pathophysiology and phenotypes. Neural cells derived from iPSCs and muscle cells derived from myoblasts can be produced in large quantity to enable drug discovery applications and facilitate translational research. Case studies will be presented for neurodegeneration (monogenic tauopathy, FTLD-tau), epilepsy disorders (Dravet Syndrome), neurodevelopmental disorders (Fragile X Syndrome, FXS), and neuromuscular disorders (Facioscapulohumeral muscular dystrophy, FSHD).

Dr. Cacace has more than two decades of bio-pharmaceutical research experience, contributing to four marketed drugs and over 18 development candidates. At Arvinas, she is leading the Neurology and Platform biology research efforts driving innovative expansion of the targeted degrader small molecule platform. Prior to joining Arvinas, Angela served as the Vice President of Biology at Fulcrum Therapeutics, where, together with her team, she built the rare disease biology platform and delivered their first development candidate for the treatment of FSHD and discovery efforts for SCD. Previously, she was the Director of Neuroscience and Genetically Defined Diseases at Bristol-Myers Squibb where she spearheaded the tau platform, alternative therapeutic modalities and was a coinventor on several development candidate patents for novel antisense oligonucleotide molecules. While at Bristol-Myers Squibb, Angela held leadership positions of increasing responsibility, building research-wide teams including centralized High Throughput DMPK Profiling, the GPCR High Throughput Screening Team and the Cellular Resource Team. While serving as a Sr. Principal Scientist in Cancer Biology at Pfizer, she was responsible for the discovery of a novel anti-angiogenic antibody development candidate. Angela received her Ph.D. in pharmacology from Columbia University and completed her postdoctoral research in Oncology at Bristol-Myers Squibb and the National Cancer Institute.

Allen Eaves, OBC, M.D., Ph.D., FRCPC

President and CEO, STEMCELL Technologies Inc.

Starting a Biotech – Going Over to The Dark Side or Seeing the Light

Dr. Eaves will briefly describe how and why he started STEMCELL Technologies, the spectrum of jobs available in a biotech, how to apply for a job in a biotech and some classic books on starting a business or working in a business.

Allen Eaves, OBC M.D. Ph.D. FRCPC, was the Founding Director of the Terry Fox Laboratory for Cancer Research for 25 years (1981-2006) and Head of Clinical Hematology at the University of British Columbia for 18 years (1985-2003). Currently Professor Emeritus of Hematology at UBC since 2006, he has devoted himself to building STEMCELL Technologies Inc, a company he founded in 1993 to provide novel and standardized tissue culture reagents for stem cell research, regenerative medicine and those doing cancer and immunological research. Always profitable, and with 97% of its sales outside of Canada, STEMCELL is Canada's largest biotech company with over 1500 employees and a global network of sales offices and distribution centres serving thousands of customers.

Dr. Eaves has published over 200 papers in leading peer-review scientific journals. He has been elected President of the International Society of Cell Therapy, President of the American Society of Blood and Marrow Transplantation, founding Treasurer of the Foundation for the Accreditation of Cell Therapy, and has been a Member of Health Canada's Expert Working Group on the Safety of Organs and Tissues for Transplantation. He has sat on the boards of The Canadian Stem Cell Foundation, The Canadian Stem Cell Network, The Centre for Commercialization of Regenerative Medicine, Mitacs, and The Banff International Research Station for Mathematical Innovation and Discovery. He is passionate about providing excellent tools and reagents for researchers and creating rewarding employment for those who love science.

Connie J. Eaves, Ph.D. FRS (Canada) FRS (Edinburgh)

Distinguished Scientist, Terry Fox Laboratory, British Columbia Cancer Research Institute; Professor, Departments of Medical Genetics, School of Biomedical Engineering, Medicine, & Pathology & Laboratory Medicine, University of British Columbia, Vancouver, BC, Canada

Normal and Malignant Stem Cells — Is Conceptual Simplicity Going Beyond Utility?

The introduction 60 years ago of functional methods to identify individual rare hematopoietic cells with stem cell properties revolutionized approaches to elucidating how many tissues with a high cell turnover maintain their numbers throughout adult life. Subsequent studies have revealed an unanticipated degree of heterogeneity in the individual molecular properties and regulation of these cells. In addition, the development of methods to rapidly and efficiently transform primary sources of human cells is now enabling these methods to be applied to elucidating the process of human leukemogenesis and breast cancer development. This talk will try to summarize the highlights of recent progress from our lab along these emerging avenues of investigation.

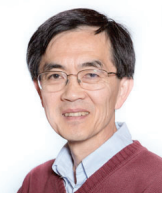
Dr. Eaves directs an internationally recognized research program in normal and cancer stem cell biology, human leukemia, and normal and malignant mammary stem cell biology. She has published more than 500 papers and has a long track record as a global scientific leader and conscientious mentor of more than100 postgraduate trainees from many disciplines. She has also been a contributor to science policy and development in Canada and abroad and has received numerous national and international awards for her many accomplishments.

Se-Jin Lee, M.D., Ph.D.

Professor, The Jackson Laboratory; Presidential Distinguished Professor; University of Connecticut School of Medicine

Myostatin: A Molecular Rheostat for Muscle Mass

Dr. Lee will discuss his work on the control of muscle growth by myostatin and a recent study in which he investigated the effect of targeting the myostatin signaling pathway in mice that were sent to the International Space Station.



Dr. Se-Jin Lee obtained his Bachelor's degree from Harvard College in 1981, graduating summa cum laude in Biochemical Sciences. He then entered the Medical Scientist Training Program at Johns Hopkins University School of Medicine, where he pursued his graduate studies with Dr. Daniel Nathans in the Department of Molecular Biology and Genetics. After obtaining his M.D. and Ph.D. degrees in 1989, Dr. Lee joined the Carnegie Institution of Washington's Department of Embryology as a Staff Associate, where he initiated his studies investigating the role of secreted proteins in regulating embryonic development and adult tissue homeostasis. Dr. Lee returned to the Department of Molecular Biology and Genetics at Johns Hopkins as a faculty member in 1991 and achieved the rank of Professor in 2001. In 2013, Dr. Lee was named as the inaugural recipient of the Michael and Ann Hankin and Partners of Brown Advisory Professorship in Scientific Innovation. In 2017, Dr. Lee moved his research program to Connecticut, where he holds a joint appointment as Presidential Distinguished Professor at University of Connecticut and Professor at The Jackson Laboratory. Dr. Lee is best known for his discovery of myostatin as a master regulator of skeletal muscle mass. In recognition of this work, Dr. Lee was elected to the U.S. National Academy of Sciences in 2012 and was awarded the Rolf Luft Award and the Ho-Am Prize in Medicine in 2013. Dr. Lee was elected as Fellow of the National Academy of Inventors in 2015.

Doug Melton, Ph.D.

Xander University Professor, Harvard and Co-Director of the Harvard Stem Cell Institute

Using Stem Cells to Make Designer Islets for Diabetics

The possibility of using stem cell-derived islets to treat insulin-dependent diabetes will be discussed. Methods for differentiating cells into normal, functional endocrine cells will be described as well as methods for genetic modification and screening to create 'designer islets' from stem cells.

Dr. Melton earned a bachelor's degree in biology from the University of Illinois and then went to Cambridge University in England as a Marshall Scholar. He earned a B.A. in history and philosophy of science at Cambridge University and remained there to earn a Ph.D. in molecular biology at Trinity College, Cambridge, and the MRC Laboratory of Molecular Biology.

Dr. Melton teaches undergraduates at Harvard College as well as graduate courses at the Harvard Medical School and Harvard Business School. He was the scientific co-founder of Gilead, Curis, and iPierian and the founder of Semma Therapeutics, now part of Vertex. He serves as a consultant to Vertex and F'Prime. Along with his wife, Gail O'Keefe, Dr. Melton served as the Co-Master of Eliot House at Harvard College from 2010-2020.

Samira Musah, Ph.D.

Assistant Professor, Duke University; Department of Biomedical Engineering Department of Medicine, Division of Nephrology; Affiliate of the Regeneration Next Initiative, Duke MEDx Investigator

Molecular and Biophysical Control of Stem Cell Fate

Members of the Musah Lab aim to understand how molecular and biophysical cues can function either synergistically or independently to guide organ development and function, and how these processes can be therapeutically harnessed to treat human disease. Research in our laboratory covers a range of interests, from fundamental studies of stem cell and tissue differentiation to engineered devices for clinical diagnostics and therapeutics. A major effort in our lab is focused on understanding the roles of molecular and biophysical cues in human organ development and how these processes can be applied to understand disease mechanisms and develop new therapeutic strategies. We develop differentiation methods by the identification and optimization of multiple, synergistic factors within the stem cell niche to guide organ-specific cell lineage specification. To engineer in vitro models of human tissues and organs, we integrate our stem cell differentiation strategies with microfluidic systems engineering, hydrogel synthesis, biofunctionalization, and three-dimensional (3D) bioprinting technologies to build dynamic circuits with living cells. Our interdisciplinary team of scientists, engineers, and clinicians use ideas and approaches spanning stem cell and developmental biology, biophysics, microengineering, chemistry, medicine, genome engineering, and computational/mathematical modeling of complex biological problems.

Dr. Samira Musah is a stem cell biologist and a bioengineer. Her work has focused on the development of novel methods to direct the differentiation of human pluripotent stem cells and engineering of microphysiological systems, including organs-on-chips and bioactive materials. She is currently an Assistant Professor at Duke University with a joint appointment in the Departments of Biomedical Engineering and Medicine. She is also a Duke MEDx Investigator and an Affiliated Faculty of the Regeneration Next Initiative. Research in her laboratory aims to understand the roles of molecular and biophysical cues in human organ development and how these processes can be harnessed to understand disease mechanisms and develop new therapeutic strategies. Dr. Musah is the recipient of numerous prestigious awards including the Whitehead Scholarship in Biomedical Research, Baxter's Young Investigator Award (top tier), Keystone Symposia Fellowship, Dean's Postdoctoral Fellowship at Harvard Medical School, Burroughs Wellcome Fund Career Transition Award, National Science Foundation Graduate Research Fellowship, Novartis Institute for Biomedical Research Award, and was named a Rising Star in Biomedical Engineering at MIT.

Alison L. O'Neil, Ph.D.

Assistant Professor, Department of Chemistry,
Department of Neuroscience and Behavior, Wesleyan University

Modeling ALS with Human Stem Cell Derived Motor Neurons

Amyotrophic Lateral Sclerosis (ALS), also known as Lou Gehrig's disease in the USA, is a terminal, fast acting, neurodegenerative disease with no cures. Motor neurons are the specific target; leaving the patient immobile and eventually unable to breathe. The O'Neil lab utilizes stem cells to access the exact cell type affected in ALS. Dr. O'Neil will present on her research using stem cell derived motor neurons to investigate potential environmental toxicants relating to the onset of sporadic ALS — which makes up 90% of cases.

Dr. Alison O'Neil received her B.S. while doing proteomics research with Prof. Anna Tan-Wilson at Binghamton University. She then went on to join the Research and Development Team at LigoCyte Pharmaceuticals (now Takeda Vaccine) working on a virus-like particle platform vaccine for Norovirus.

In 2008, she joined the Chemistry and Biochemistry department at Montana State University and earned her Ph.D. under Prof. Trevor Douglas. At MSU, she was awarded the Graduate Excellence award for the class of 2013. Her Ph.D. research exploited the self-assembling properties of bacteriophage P22 to engineer nanomaterials.

During her post-doctoral studies, Dr. O'Neil worked on modeling and understanding neurodegenerative diseases using human stem cells at Harvard University and the Broad Institute of MIT and Harvard. This work included projects involving Parkinson's disease, ALS, and schizophrenia. While at Harvard, she authored collaborative work with Google, Vertex, and Biogen Idec.

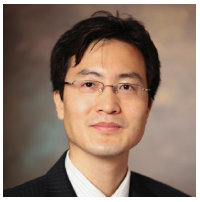
Joining Wesleyan in the summer of 2018, her lab's research combines her love of protein biochemistry and stem cell training by investigating the role of protein aggregates in the progress of neurodegeneration.

In-Hyun Park, Ph.D.

Associate Professor, Department of Genetics and in the Child Study Center,
Yale University

Generation of Integrated Brain Organoids to Investigate Brain Development and Diseases

Human brain organoid techniques have rapidly advanced to facilitate investigating human brain development and diseases. Since the first report, a number of protocols were reported to produce brain organoids, raising a question whether the brain organoids from different protocols are similar or different. We accrued the scRNA-seq data from the published works, and performed a comparative analysis. We found that regardless of methods, brain organoids produce similar types of cells that are produced in primary brain. We also applied the brain organoids tools to investigate human brain developmental disorders, called Rett syndrome (RTT) that are caused by mutations in MeCP2. We derived hESC lines with mutations in MeCP2 by CRISPR-editing, and applied the genomics tools to neurons and brain organoids derived from the MeCP2 mutant cells. We found that MeCP2 mutations caused the neural dysfunction by the abnormal transcription regulation, and that BET inhibitor JQ1 rescued the phenotypes of MeCP2 null neurons and animal. Overall, our studies demonstrated that brain organoids are important tools that can be readily used to study human brain development and diseases.



Dr. Park is an Associate Professor of Genetics, Yale Stem Cell Center, and Child Study Center. Dr. Park received his B.S and M.S. from Seoul National University at Korea, and Ph.D. from University of Illinois at Urbana-Champaign in the field of Cell and Structural Biology. During his Ph.D. training with Dr. Jie Chen, he studied mTOR pathways regulating cell growth, and myogenic differentiation. In 2005, he continued his research as a Post-doc fellow in Dr. George Daley's lab in Children's Hospital Boston, where he isolated one of the first human induced pluripotent stem cells (iPSCs). He started his own lab at Yale University from 2009. As an independent investigator, he pioneered in the generation of region-specific brain organoids, and study the human neurodevelopment and related disorders.

Karolina Palucka, M.D., Ph.D.

Professor; Associate Director for Cancer Immunology; Deputy Director, JAX Cancer Center; The Jackson Laboratory for Genomic Medicine

Human-Derived Ex Vivo Systems for Studies on Lung Immunity to Respiratory Viruses

Dr. Palucka will discuss the generation of 3D lung epithelial tissues from organoids and their use for studies of viral responses including influenza virus and SARS-CoV-2 virus.

Karolina Palucka, M.D., Ph.D. is a Professor and Associate Director of Cancer Immunology at The Jackson Laboratory for Genomic Medicine (JAX) in Farmington, CT and also serves as a Professor in the Department of Immunology at the University of Connecticut School of Medicine. She joined JAX in 2014; having previously been at the Baylor Institute for Immunology Research, where she was the Michael A.E. Ramsay Chair for Cancer Immunology Research and director of the Ralph M. Steinman Center for Cancer Vaccines. Dr. Palucka also served as a professor at the Mt. Sinai School of Medicine within the Department of Gene & Cell Medicine. She received her M.D. from the Warsaw Medical Academy, Poland and her Ph.D. from the Karolinska Institute in Sweden. Dr. Palucka specializes in human immunology, with a focus on experimental immunotherapy, and has pioneered the development of dendritic cell-based vaccines for patients with cancer or HIV. Her research is aimed at understanding, controlling, and manipulating the body's own immune response as the basis for developing new vaccines and immunotherapies against infectious diseases and human cancers.

Michael Bronson Ph.D.

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10x Genomics develops tools to enable high-throughput single-cell genomics and multi-omics technologies. Here we will share new capabilities we're launching in 2021 to increase resolution by studying more dimensions and simultaneous measurements providing complete context — increased scale to study more cells, more tissues, more samples. We will also highlight some of the 10x enabled discoveries that shape our understanding of the cellular, molecular, and temporal mechanisms underlying regeneration, cell-type specification, and organ formation, giving us a complete picture of development.

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Michael Bronson, earned a Ph.D. from Brown University studying transcription and translational response to Estrogen in breast cancer. Has over 10 years of experience, including field application scientist and business development positions in precision medicine and bioinformatics. Joined 10x Genomics in 2017, now a Senior Sales Executive providing support for multi-omic single-cell genomics and the multi-omic spatial analysis of intact tissues for academia and industry clients in NYC and CT.

Diane Krause M.D., Ph.D. *(Organizing Chair) Department of Cell Biology, Department of Laboratory Medicine, Department of Pathology, Associate Director, Yale Stem Cell Center, Yale University*

Gordon Carmichael, Ph.D. *Department of Genetics and Genome Sciences, UCONN Stem Cell Institute, University of Connecticut Health Center*

Stormy Chamberlain, Ph.D. *Department of Genetics and Genome Sciences, UCONN Stem Cell Institute, University of Connecticut Health Center*

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Shangqin Guo, Ph.D. *Department of Cell Biology, Yale Stem Cell Center, Yale University*

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Milton Wallack, D.D.S. *Connecticut Stem Cell Research Coalition, Connecticut Regenerative Medicine, Research Advisory Committee*

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