

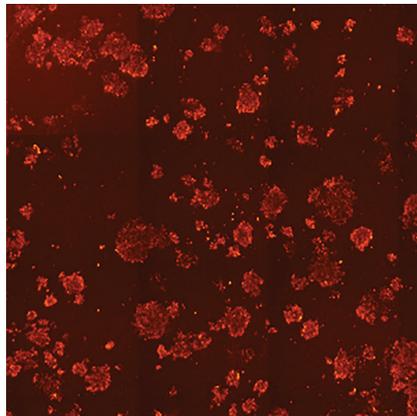
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## STEM CELLS FROM A FEW DROPS OF BLOOD



Blood cell-derived induced pluripotent stem cell colonies

A team of NYSCF scientists led by first author **Dr. Monica Zhou** developed a method of creating induced pluripotent stem (iPS) cells from very small amounts of cryopreserved umbilical cord blood within two weeks in a cost effective, efficient, and scalable way. Further, the method does not compromise the DNA integrity of the derived cells, a critical component as damaged DNA can lead to cancer.

This advance expands the pool of potential patients who can participate in and benefit from stem cell research. "Pediatric patients like babies and children who previously could not give large blood or skin samples can now be a part of personalized medicine," **Dr. Scott Noggle**, NYSCF Vice President of Stem Cell Research and co-author of the paper, noted. "With this opportunity to create stem cells from children and others, we can investigate disease, test drugs, and one day create transplants for patients of all ages."

Blood remains the most accessible way to study what goes on beneath a patient's skin. With this recent publication in *Stem Cell Reviews and Reports*, scientists at the NYSCF Research Institute developed a quick and safe method to transform easily collected and stored blood samples into iPS cells for current and future research.

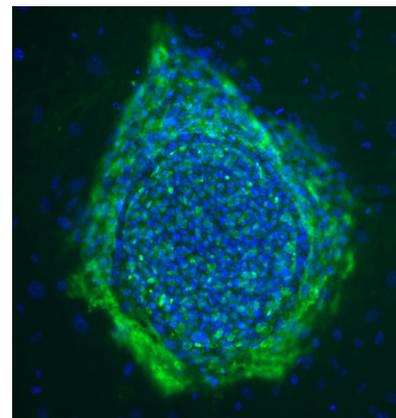
**Dr. Joanne Kurtzberg**, Director of the Pediatric Blood and Marrow Transplant Program at Duke University and a co-author of the paper, coordinated the use of cord blood samples from young children suspected to be affected by genetic diseases for this important research. Prior to this method, the limited amount of cells in umbilical cord blood samples typically reserved them for therapeutic uses, restricting access for researchers. Through this technique developed at NYSCF, researchers can now model diseases, look for genetic mutations leading to sickness, and examine the cellular relationships between molecules using cord blood samples, bringing the power of stem cells to a new spectrum of patients.

## NYSCF AND THE BROAD INSTITUTE PARTNER TO FIND BETTER TREATMENTS FOR PSYCHIATRIC DISORDERS

NYSCF is creating the world's largest collection of stem cell lines from patients with schizophrenia and other psychiatric diseases in collaboration with the **Stanley Center for Psychiatric Research at the Broad Institute** of Harvard and MIT. This resource will allow scientists to study the causes of disease on the affected cells and enable researchers to discover and test new medicines. **Dr. Steven Hyman**, Director of the Stanley Center for Psychiatric Research at the Broad Institute stated, "In the short term, [this] will help us better understand misunderstood and difficult condition[s]. In the longer term, it will help provide important information and approaches for drug discovery."

The partnership will bring researchers closer to understanding why certain patients develop psychiatric diseases and reveal which brain cells are involved and how they are affected. The research made possible by this resource will inform the development of future stem cell therapies and the discovery of new treatments through testing potential drug candidates in a dish.

The pioneering **NYSCF Global Stem Cell Array™** robotic technology will be used to create stem cell lines from skin samples from patients with schizophrenia supplied by the Stanley Center. NYSCF scientists will then manipulate these stem cells to mature into brain cells to illuminate how psychiatric disease alters different brain tissue. The Array technology makes this partnership possible by providing the ability to automate the creation of a vast number of quality standardized stem cell lines.



Stem cells produced by NYSCF Global Stem Cell Array™

## LETTER FROM THE CEO



Dear Friends,

The first six months of 2015 have already been eventful at the NYSCF Research Institute with new partnerships announced, multiple major publications in top journals, exciting new programs and initiatives, and the continued expansion of our international scientific community.

We have built a global network of over 120 of the top stem cell researchers, bioengineers and neuroscientists whose work is consistently recognized in the most prestigious scientific journals and publications. At the NYSCF Research Institute, we are accelerating the pace of discovery with the one-of-a-kind NYSCF Global Stem Cell Array technology. As you will see throughout this newsletter, our capabilities and reach continue to grow and broaden.

Our collaborative model has never been more important. In the past year alone, NYSCF spoke in London, Argentina, Stockholm, and China, as well as across the United States sharing our innovative research model and breakthrough discoveries. By pursuing high risk/high reward research and bridging the gaps between academia, government, disease foundations, and industry, the NYSCF Research Institute and our collaboration partners create unprecedented research results.

In addition to bench research, we tackle large-scale problems through NYSCF's unique ability to convene groups of diverse, cross-specialty, and international stakeholders. One such initiative this year involved building an Immunoengineering working group of leading stem cell scientists, big data technologists, bioengineers, and immunologists to address the autoimmune response in type 1 diabetes and other autoimmune disorders. In addition, we convened the Global Alliance for iPSC Therapies, or GAI<sup>T</sup>; an international coalition of stakeholders from eleven different countries negotiating a framework for providing future iPSC therapies to the global population.

In the past ten years as the science, technology, and bioengineering fields have grown exponentially, NYSCF is playing a leading role in precision medicine by conducting and accelerating global research efforts to find the cures for the major diseases of our time.

As we reflect on the past ten years and look forward to the future, we recognize that every new breakthrough, every paper published, and every initiative that brings us closer to cures is a direct result of your commitment to our mission. We could not have come this far without your help.

With your continued support, we are ready to begin bringing this research to the clinic to treat patients. Stay with us over the next ten years. The treatments we are developing across multiple diseases are nothing short of miraculous.

On behalf of all of us at NYSCF I thank you from the bottom of my heart.

With warm regards,

Susan L. Solomon

## CLIMB FOR HOPE



Team NYSCF on Mount Rainier. *From left:* James Teague, Mark McCauley, Dieter Egli, PhD, and Rich Rundle.

This summer, team NYSCF returned to raise awareness and give hope to those suffering from chronic and degenerative diseases by scaling Mount Rainier in a four-day climbing effort. Joining forces on the **Climb for Hope** were NYSCF scientist **Dr. Dieter Egli**, Junior Leadership Council Board Members **Mark McCauley** and **Rich Rundle**, and NYSCF supporter **James Teague**. The team successfully summited Mt. Rainier, the highest peak in the Pacific Northwest, climbing over 14,400 feet on their philanthropic mission.

Last year, in the first effort of its kind for NYSCF, Rich Rundle organized the Ride For Hope to support the NYSCF Research Institute's work to find cures for the major diseases of our time including multiple sclerosis, a cause close to him as his brother, Matt, suffers from the disease. The Ride For Hope consisted of an intensive, two-day, 270 mile bike ride from the NYSCF Research Institute in Manhattan to the steps of the Capitol Building in Washington, D.C. Dr. Egli completed the two man team on the ride to raise awareness for stem cell research and hope for all patients.

Building on the momentum of last year's success, team NYSCF and all of NYSCF's supporters made the 2015 Climb For Hope a huge success surpassing this year's fundraising goal of \$25,000 by raising \$32,515. In total, over twice as much as was raised last year.



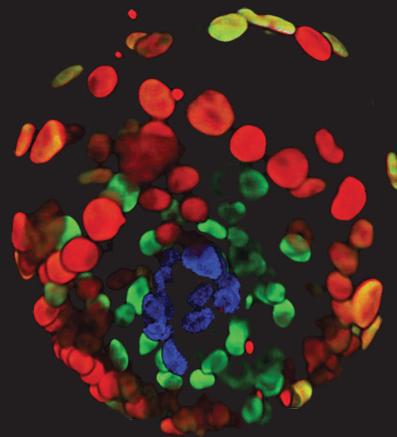
*From left:* Mark McCauley, James Teague, and Rich Rundle during the Climb For Hope

THE NEW YORK STEM CELL FOUNDATION RESEARCH INSTITUTE

# 10<sup>th</sup> Anniversary Gala Celebration

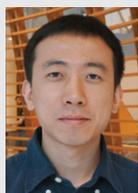
TUESDAY, OCTOBER 20, 2015

SKYLIGHT AT MOYNIHAN STATION



## THE 2015 CLASS OF NYSCF – DRUCKENMILLER FELLOWS

NYSCF welcomes 3 new NYSCF – Druckenmiller Postdoctoral Fellows to the NYSCF Fellowship Program. These 3 outstanding up-and-coming scientists join 11 current Fellows and 38 alumni who have published over 575 papers in top journals.



**Hongda Li**  
Rockefeller University

Dr. Li employs stem cells to study the function of enzymes present at the final stage of cell division providing insight into the machinery controlling the cell cycle and brain development in humans under the guidance of Dr. Joseph G. Gleeson at Rockefeller University.



**Brett Shook**  
Yale University

Dr. Shook focuses on fat cells and their potential role in skin repair as a novel therapy for wound treatment in Dr. Valerie Horsley's laboratory at Yale University.



**Dafne Campigli Di Giammartino**  
Weill Cornell Medical College

Dr. Di Giammartino explores the architecture of condensed DNA in stem cells in Dr. Effie Apostolou's laboratory at Weill Cornell Medical College.

## ANNUAL NYSCF – DRUCKENMILLER FELLOWS RECEPTION

May marked the initiation of the 2015 NYSCF – Druckenmiller Fellows. NYSCF gathered at the Asia Society in Manhattan to welcome the new Fellows to the extraordinary NYSCF Innovator community.



*Top row from left:* Maurie Perl, Peggy Bewkes, and Beverly Hurwitz. Dafne Campigli Di Giammartino, PhD, Stan Druckenmiller, Brett Shook, PhD, and Hongda Li, PhD. Susan L. Solomon and Fiona Druckenmiller. *Bottom row from left:* Sandra Lloyd, her grandson, and Dieter Egli, PhD. Paul Goldberger and John Elderfield. Scott Noggle, PhD, and Bonnie Pfeifer Evans.

## IMMUNOENGINEERING: NYSCF CONVENES GLOBAL BRAIN TRUST TO TACKLE AUTOIMMUNE DISEASE



The Immunoengineering Working Group brainstorms new approaches for type 1 diabetes

NYSCF convened a groundbreaking meeting on **Immunoengineering** to devise novel approaches for finding treatments and cures for type 1 diabetes and other autoimmune disorders. The meeting, co-chaired by **Susan L. Solomon**, NYSCF CEO and Co-founder; **Dr. Ed Boyden**, MIT Professor of Biological Engineering, Brain and Cognitive Science; **Sir John Bell**, Regius Professor of Medicine, Oxford University; and **Dr. Doug Melton**, Co-Director of Harvard Stem Cell Institute, included experts in immunology, bioengineering, mathematics, data science, nanotechnology, and more, and focused on generating new ideas and combination approaches beyond cell replacement to address the autoimmune root of type 1 diabetes. The group of experts, from tech companies, non profits, and universities worldwide, evaluated the current methods for targeting and tempering human immune responses to create new frameworks for pursuing treatments.

to guide the body in an elegantly orchestrated attack against the invaders. However, sometimes the body's natural defense mechanism goes awry, becoming hyper-vigilant and attacking itself. This overreaction leads or contributes to many diseases including diabetes, multiple sclerosis, arthritis, celiac disease, and other disorders. Aiming to develop new viable treatments under FDA guidelines, the Immunoengineering Working Group will continue and expand discussions following the success of this first-of-its-kind meeting. NYSCF's Immunoengineering Working Group looks to answer what causes the immune system to turn against the body and therefore cure or prevent these devastating diseases.

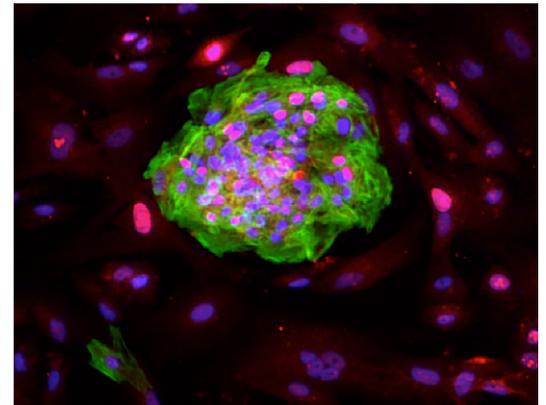
Current type 1 diabetes treatments fail to address the immune attack on a patient's insulin-producing cells. Functioning immune systems operate by sensing foreign agents in the body, which then trigger the recruitment of killer cells and defensive molecules

## REPAIRING THE INJURED HEART

NYSCF Principal Investigator **Dr. Danny Freytes** and his team brought patient-specific heart repair patches one step closer to clinics by studying the interactions between stem cell-derived heart muscle cells (cardiomyocytes), and surrounding inflammatory cells. Heart attacks kill cardiac tissue. To treat this damage, scientists envision patching damaged hearts with new heart cells made from patient-specific stem cells. To bring heart patches to the clinic, Dr. Freytes works to understand how the cells his team creates in the NYSCF Research Institute will interact with damaged heart cells in a patient.

The NYSCF team published their latest research uncovering the ways these lab-grown cardiomyocytes communicate with other cells in the *Journal of Tissue Engineering and Regenerative Medicine*. Modeling heart injury in a petri dish, the scientists examined how proteins signaled to one another between the stem cell-derived cardiomyocytes and macrophages — a type of immune cell — to shed light onto how these interactions can be harnessed to design successful therapies.

“As we learn how cardiac repair cells grown in the lab recruit and interact with macrophages during wound healing, and how in turn macrophages affect the repair cells, we gain important insights into the complex cross-talk that occurs between our engineered constructs and the damaged heart,” Dr. Freytes explained. Before bringing this research to patients, scientists need to further ensure that their patches survive, embed themselves in patient's hearts, and successfully restore function.



Stem cell-derived cardiomyocytes



## 10<sup>th</sup> ANNUAL TRANSLATIONAL STEM CELL RESEARCH CONFERENCE

Casparry Auditorium, The Rockefeller University, New York City

OCTOBER 28 - 29, 2015

TO REGISTER GO TO  
[WWW.NYSCF.ORG/CONFERENCE](http://WWW.NYSCF.ORG/CONFERENCE)

## CREATING A GLOBAL NETWORK OF STEM CELL BANKS

Representatives from leading research organizations in eleven different countries gathered at a NYSCF-hosted meeting in New York City to discuss the formation and structure of the **Global Alliance for Induced Pluripotent Stem Cell (iPSC) Therapies, or GAIT**. This alliance will create an international system of stem cell banks and organizations with a goal of making future iPSC therapies available to the global population. This key resource will provide patients around the world with access to standardized stem cells compatible with their immune systems through a model similar to blood banks in anticipation of a global demand for personalized medicine.



NYSCF hosts the Global Alliance for iPSC Therapies (GAiT) in New York City

The GAIT mission to make iPSC therapies available to all patients in need closely aligns with the NYSCF mission of accelerating treatments and cures for the major diseases of our time. As a founding member of this global alliance, NYSCF's capabilities, including the NYSCF Global Stem Cell Array™ technology, may play a crucial role as the international GAIT network continues to develop.

## NYSCF INNOVATOR COMMUNITY GATHERS AT ANNUAL RETREAT

At NYSCF's Seventh Annual Innovators Retreat, NYSCF – Druckenmiller Fellows, NYSCF – Robertson Investigators, and NYSCF Research Institute scientists gathered to discuss the latest in stem cell and neuroscience research. The Retreat gives the NYSCF scientific community the chance to explore each other's research and discoveries as well as share unpublished data and findings to foster cutting-edge collaboration in tackling disease.

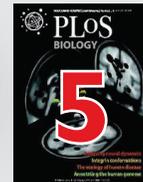
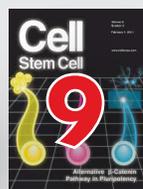
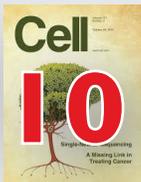


From left: NYSCF innovators discuss collaboration. Chris White, PhD, former DARPA program manager, gives keynote address on using big data to optimize research. NYSCF innovator community gathers at the annual retreat.

## INNOVATORS AT A GLANCE

The NYSCF Innovator community of outstanding research scientists is comprised of the NYSCF – Druckenmiller Postdoctoral Fellows, NYSCF – Robertson Early Career Investigators, and NYSCF Research Institute scientists from around the world. Our community of over 120 preeminent scientists continues to conduct the most cutting-edge stem cell, neuroscience, and bioengineering research bringing us closer to a future of personalized medicine to eliminate the major diseases of our time.

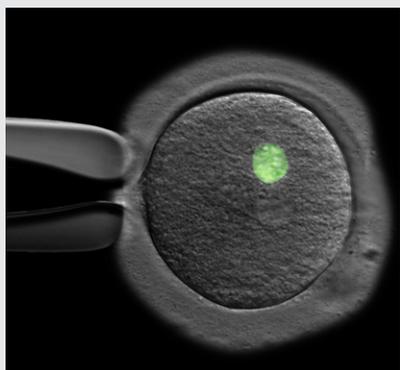
### NYSCF INNOVATOR RESEARCH IN TOP JOURNALS SINCE 2014



**150+** = # of papers published by NYSCF Innovator community since 2014

**480+** = # of times papers cited in top peer-reviewed journals in the past year

## ADVOCATING FOR LIFESAVING TREATMENT



DNA transferred into a healthy donor oocyte (egg cell)

This spring, the United Kingdom voted to approve clinical trials for the use of mitochondrial replacement therapy, or MRT, while the United States continues to lag behind. This disease-preventing technique, developed in part by NYSCF researchers, still requires approval by the Food and Drug Administration (FDA) before NYSCF can bring this treatment to patients in the United States.

Speaking to the Institute of Medicine (IOM) during public comments in March, NYSCF Chief of Staff, **David McKeon**, explained that about every 30 minutes a child in the U.S. is born with a mitochondrial disease and the technology exists today to prevent their inheritance. The FDA asked the IOM to weigh the ethical and societal concerns for this new technique, which will prevent mitochondrial diseases in future generations.

Mitochondria function as cellular batteries. People born with malfunctioning mitochondria experience a spectrum of symptoms potentially resulting in childhood death, including stunted development, neurological disorders, heart disorders, and stomach and digestive problems.

Mitochondrial diseases remain incurable. But, in developing nuclear transfer techniques, NYSCF scientists pioneered a method to avoid passing mutated mitochondria to the next generation. Removing the nuclear DNA from the eggs of hopeful mothers with mitochondrial disease, researchers transfer the DNA into healthy donor eggs that have had their DNA removed.

As NYSCF scientist **Dr. Dieter Egli** explained to the *New York Times Magazine* in June 2014, “This technique does not create designer babies and does not enable scientists to modify genes. MRT simply allows affected families to choose to have unaffected children.”

NYSCF, on the cutting-edge of discovery, advocates to bring this technique to the public. **Susan L. Solomon**, NYSCF CEO, wrote in *Roll Call*, a leading Washington D.C. political journal: “The women who are suffering from these diseases and are at risk of passing them on to their children deserve the autonomy and the right to make their own informed decisions on how they will build their families. With the advent of new technologies, including MRT, it is time for the US to once again be a world leader in offering our citizens the best medical care.”

## WORKING TOWARDS GENDER EQUALITY

As NYSCF's voice in national conversations on scientific innovation expands, NYSCF works to take the lead in another national discussion—closing the gender gap in science and engineering. NYSCF's **Initiative on Women in Science and Engineering (IWISE)** provides a roadmap toward gender equality.

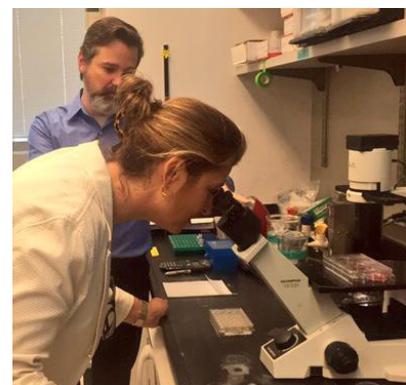
The IWISE Working Group published recommendations to promote gender equality within science and engineering in *Cell Stem Cell*. In the 2<sup>nd</sup> annual meeting, men and women in various career stages gathered to devise practical strategies, including many that require no funding to implement: inviting women to sit on committees, debunking biases with implicit bias statements, and creating opportunities to educate i.e. gender awareness training.

Leading by example, NYSCF incorporates many IWISE recommendations. Specifically, NYSCF works toward using IWISE's Institutional Report Card for Gender Equality when considering external grants.



IWISE participants discuss actionable steps to promote gender equality

The report card holds institutions accountable for equitable gender practices. Sifting through items such as the proportion of females in departments, hiring history, and family-flexibility policies to set a grade, NYSCF hopes to make this rubric an integral part of grant applications across the field. As NYSCF and other grant-making organizations incorporate this new standard, IWISE envisions equitable gender representation as the norm.



NYSCFVP, Stem Cell Research Dr. Scott Noggle and Deputy Mayor Alicia Glen

### NEW YORK CITY: A GROWING BIOTECH HUB

New York City Deputy Mayor for Housing and Economic Development **Alicia Glen** and her colleague **Carolee Fink** visited the NYSCF Research Institute in June. The Mayor's Office continues to recognize outstanding science in the city and grows the city's status as a biotechnology hub.

## NYSCF SPEAKS AROUND THE WORLD



Susan L. Solomon debates genetic engineering and the future of stem cell research at the 92nd Street Y. *From left:* Dr. Klitzman, Susan L. Solomon, Drs. Metz, Silver, and Church

NYSCF CEO, **Susan L. Solomon**, debated stem cells and the future of genetic engineering as part of an expert panel confronting humanity's future beyond science fiction. The **92nd Street Y** panel, moderated by **Dr. Jamie Metz** and entitled *Homo Sapiens 2.0: Genetic Enhancement and the Future of Humanity*, featured Ms. Solomon, **Dr. George Church**, Professor of Genetics at Harvard Medical School; Columbia bioethicist **Dr. Robert Klitzman**; and **Dr. Lee Silver**, Princeton Professor of Molecular Biology and Public Policy.

**Dr. Stephen Chang**, NYSCF Vice President of Research and Development, spoke about modeling diseases using stem cells and the NYSCF Global Stem Cell Array™ Technology at the **National Institutes of Health** during the 5<sup>th</sup> American Institute for Medical and Biological Engineering Workshop on Validation and Qualification of New In Vitro Tools and Models for the Pre-clinical Drug Discovery Process.

The 11<sup>th</sup> Annual Alzheimer's Disease Education Conference and Expo presented by the **Alzheimer's Disease Resource Center** invited NYSCF Vice President of Stem Cell Research **Dr. Scott Noggle** to share the latest in NYSCF's Alzheimer's Disease research and highlight the importance of stem cells in studying neurodegenerative disease.

During the **Days of Molecular Medicine** conference at Sweden's **Karolinska Institutet**, the awarding institution of the Noble Prize in Physiology or Medicine, Ms. Solomon shared NYSCF's model for collaborative research in addition to serving on the Program Committee. In addition, Ms. Solomon gave a keynote interview entitled, "Realizing the promise: How the NYSCF is enabling the acceleration of cures through stem cell research," at the **10<sup>th</sup> Annual World Stem Cells and Regenerative Medicine Congress** in London.



The NYSCF team attending the International Society for Stem Cell Research annual meeting in Stockholm, Sweden

NYSCF once again had a large presence at the **International Society for Stem Cell Research** conference in Stockholm, Sweden to learn and share the latest in stem cell advances worldwide. NYSCF scientist **Dr. Mitsutoshi Yamada** presented on advances in curing mitochondrial disease using nuclear transfer.

### NYSCF JUNIOR LEADERSHIP COUNCIL EVENT AT BOWERY HOTEL

The NYSCF Junior Leadership Council, a group of young professionals committed to advancing medicine, act as NYSCF ambassadors increasing awareness for NYSCF's work through outreach and financial support. As part of the NYSCF family, members learn about the latest breakthroughs and mingle with researchers at events throughout the year.



*From left:* Adam Stone, Todd Stone, Christine Lin, Sabrina Bertucci, and Brian Campos. Kyle McCauley, Lauren Massalas, and Mark McCauley. Matt Quarello, Amber Emery, Brett Thomas, and Brian Trotta.

**NYSCF Webinar Series** – *Translating Research into Cures*

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# NYSCFNEWSupdate

## NYSCFNEWSupdate

### FIVE REASONS TO SUPPORT THE NEW YORK STEM CELL FOUNDATION

1. We focus on high-risk, high-reward ideas that traditional funding mechanisms won't support.
2. We have a proven track record of identifying, initiating and funding critical "tipping-point" experiments.
3. We make possible the experiments that are changing the landscape of stem cell science.
4. We are independent and have no political or institutional agenda – and always put the best science first.
5. We focus **ONLY** on translational research for patients.

### HOPE FOR THE FUTURE STARTS NOW!

Your generosity helps support stem cell research.  
Donate directly on-line at [www.nyscf.org/donate](http://www.nyscf.org/donate) or mail a check to:

The New York Stem Cell Foundation  
1995 Broadway, Suite 600, New York, NY 10023

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**NYSCF'S MISSION IS...** to accelerate cures for the major diseases of our time through stem cell research.

#### *Our Programs*

- **NYSCF Research Institute:** Pursuing the most advanced stem cell research in our own private laboratory, we develop unique stem cell technology that makes precision medicine a reality.
- **NYSCF Innovators:** Supporting and training the next generation of scientists doing innovative high-risk/high-reward work that translates research into cures and annually honoring the most significant achievement in stem cell research by an early career scientist.
- **NYSCF Outreach:** Convening the world's preeminent annual translational stem cell research conference and an ongoing series of programs for scientists, policymakers and the public to provide unbiased expert access to life-changing information.