NYSCF
The New York Stem Cell Foundation Research Institute

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# NEWSupdate

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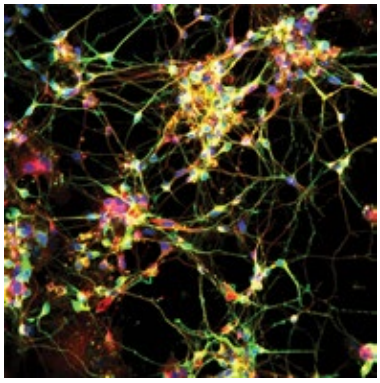
## NYSCF STUDIES IDENTICAL TWINS: UNRAVELING THE MYSTERY OF PARKINSON'S DISEASE


NYSCF scientists successfully created a human stem cell model of Parkinson's disease in a dish using skin cells from a pair of identical twins, one affected and one unaffected with Parkinson's disease. The results identified potential therapeutic targets for Parkinson's disease specifically related to the twins' neurons' ability to produce dopamine, the neurotransmitter molecule that is deficient in Parkinson's disease, which causes the disease symptoms. This effort gave the researchers an unprecedented opportunity to evaluate and dissect the genetic and non-genetic contributions to disease development.

Described in the cover story of *Cell Reports* on November 6th, a team led by NYSCF scientists **Dr. Scott Noggle**, **Dr. Aiqun Li**, **Chris Woodard**, and **Brian Campos** made induced pluripotent stem (iPS) cells from skin samples from both twins to generate a cellular model of Parkinson's in a dish. Using these cell models, the scientists were able to observe key features of the disease.

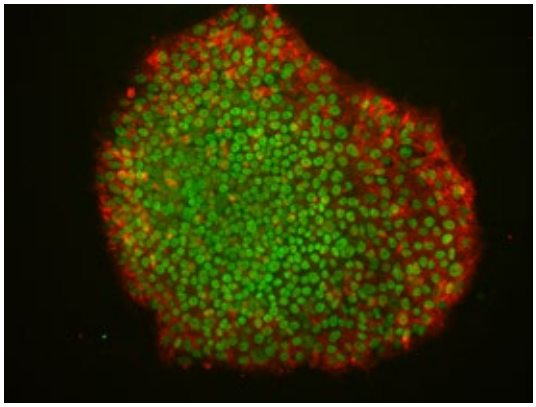


*Parkinson's disease neurons*

Attributed to a combination of genetic and nongenetic factors, Parkinson's disease has no completely effective treatment or cure. Parkinson's disease is moderately heritable, but the mechanisms of this inheritance are not yet well understood. While genetic forms of the disease exist, idiopathic forms of the disease, not associated with a known genetic mutation, are far more common. Over one million Americans and ten million people worldwide are affected by this progressive neurodegenerative disorder.

In NYSCF's research, genetic and stem cell analysis identified a potentially useful combination therapy that may be applicable to all Parkinson's disease patients. While this case study is unique, this research model and cellular analysis could yield further clues to all cases of genetic and idiopathic Parkinson's disease and other related neurological disorders.

## ONE STEP CLOSER TO CELL REPLACEMENT THERAPIES FOR MULTIPLE SCLEROSIS PATIENTS



*Multiple sclerosis induced pluripotent stem cells*

Scientists at The NYSCF Research Institute are one step closer to creating a viable cell replacement therapy for multiple sclerosis from a patient's own cells. For the first time ever, NYSCF scientists made induced pluripotent stem (iPS) cells from skin samples of patients with primary progressive multiple sclerosis, the most aggressive form of the disease.

In this significant advance, a team of scientists led by **Dr. Valentina Fossati,** NYSCF – Helmsley Investigator, developed a dramatically faster method of coaxing these stem cells into becoming oligodendrocytes, the myelin-forming cells of the central nervous system that are affected in multiple sclerosis and many other neurological and central nervous system diseases.

Existing techniques for making oligodendrocytes take almost half a year to complete, limiting the ability of scientists to conduct their research. This study, published in *Stem Cell Reports* on July 24th, demonstrates that the time needed to make these cells can be cut in half, making it much easier to use these cells in research, disease modeling, and drug screening. While cell replacement therapies are still likely years away from the clinic, this research demonstrates that scientists are one step closer to replacing the damaged cells in the brain with healthy cells generated using this method.

# LETTER FROM THE CEO

Dear Friends,

2014 was highlighted by the exceptional progress NYSCF Research Institute scientists have made in finding cures for the most devastating diseases of our time. This summer, we developed a revolutionary new protocol to make patient and disease-specific neurons for **multiple sclerosis**, pushing us ever closer to new cell replacement therapies and better drugs for this devastating and currently incurable disease. In addition, we conducted a unique **Parkinson's disease** study with identical twins, only one of whom is affected by the disease, which identified potential therapeutic targets for Parkinson's disease. In a landmark study, we also compared the chracteristics of two different methods of deriving stem cells. This fall, we launched the NYSCF Stem Cell Repository, which will be the largest ever stem cell repository and resource for scientists around the world.

Since NYSCF's inception, we have awarded 30 NYSCF – Robertson Investigator awards to exceptional stem cell researchers and neuroscientists, and 47 NYSCF – Druckenmiller Postdoctoral Fellowship awards to promising young researchers. All of these researchers, together with the 45 scientists in the NYSCF Laboratory, comprise NYSCF's global scientific community of over 120 of the world's preeminent stem cell scientists who are defining the field as it moves forward.

The NYSCF model of collaboration and bridging the gap between institutions continues to accelerate research. To date, NYSCF has partnered with over 50 leading disease foundations, academic institutions, pharmaceutical companies, and government entities. Two significant recent collaborations are NYSCF's multi-stakeholder effort with the Beyond **Batten Disease** Foundation to find cures and treatments for juvenile Batten disease, a fatal childhood illness, and an effort with the **Charcot-Marie-Tooth** Association to research multiple forms of this inherited and progressive group of diseases.

None of NYSCF's achievements would be possible without your steadfast and ongoing support. As we head into our tenth anniversary year, we reflect back on how much we have accomplished and look ahead to how much more there is to do. I hope you will join us in our search for cures in 2015 and beyond. Together, we can push science forward and bring exciting research discoveries from the lab to the clinic.

Warmest wishes for the holidays and a happy and healthy new year,

Susan L. Solomon

**Please donate to NYSCF by December 31 to take advantage of your 2014 tax deduction!**

***nyscf.org/donate***

**To make gifts of appreciated stock or wire transfers:**

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*We depend on your support!*

# THE 2014 NYSCF – ROBERTSON INVESTIGATOR CLASS ANNOUNCED

On October 14th, The New York Stem Cell Foundation named six outstanding scientists as the **2014 NYSCF – Robertson Investigators.** The NYSCF – Robertson Stem Cell and Neuroscience Investigator programs are regarded among the most prestigious stem cell and neuroscience awards in the world. Successful candidates complete a rigorous application and review process conducted by a jury of the best minds in the field.

This year's six new NYSCF – Robertson Investigators, together with the 26 existing Investigators, join the 47 NYSCF – Druckenmiller Postdoctoral Fellows and the 45 scientists at the NYSCF Laboratory to form NYSCF's scientific community: a network of over 120 scientists at leading institutions worldwide.

Each NYSCF – Robertson Investigator will receive a $1.5 million award, disbursed over the next five years, to advance innovative research by expanding his or her laboratory, and training other scientists.

The **2014 NYSCF – Robertson Stem Cell Investigators:**

**Valentina Greco**, **PhD**, Yale University. Dr. Greco utilizes genetic, live imaging, and genomic approaches to capture the emergence of cancer in order to transform current therapeutic strategies and find new cures and prevention strategies.

**Jennifer Phillips-Cremins**, **PhD**, University of Pennsylvania. Dr. Phillips-Cremins focuses on understanding the mechanisms that govern the production of healthy neurons from stem cells and how these mechanisms go awry in neurodegenerative diseases.

**Feng Zhang**, **PhD**, Broad Institute of MIT and Harvard and Massachusetts Institute of Technology. Dr. Zhang is developing and applying disruptive technologies like optogenetics and genome engineering to understand nervous system function and disease.

The **2014 NYSCF – Robertson Neuroscience Investigators:**

**Edward Chang**, **MD**, University of California, San Francisco. Dr. Chang specializes in advanced clinical brain mapping methods to safely perform neurosurgical procedures in eloquent areas of the brain.

**Lisa Giocomo**, **PhD**, Stanford University School of Medicine. Dr. Giocomo integrates a variety of disciplines and tools to study how single-cell biophysics and network dynamics interact to mediate spacial memory and navigation.

**Kay Tye**, **PhD**, Massachusetts Institute of Technology. Dr. Tye focuses on understanding how the brain processes the differences between positive and negative stimuli and using cutting-edge techniques to induce long lasting behavior changes.

# THE NYSCF MODEL OF ACCELERATING CURES

***NYSCF has developed a unique model of research by collaborating and building infrastructure, resources, and new technologies to accelerate cures for the major diseases of our time. By breaking down barriers and bridging the gaps between academia, government, disease foundations, and the private sector, NYSCF accelerates research on a global scale.***

## PARTNERSHIP TO FIND A CURE FOR JUVENILE BATTEN DISEASE

NYSCF and the **Beyond Batten Disease Foundation (BBDF)** are developing stem cell resources to discover new treatments and cures for juvenile Batten disease, a fatal childhood illness.

NYSCF scientists are creating induced pluripotent stem (iPS) cell lines from skin samples of young people affected by the disease as well as unaffected family members. Reprogramming these iPS cells to become brain and heart cells will provide the infrastructure needed to investigate the disease. The cell lines created will be the largest and first genetically diverse collection of human iPS cells for a pediatric brain disease.

NYSCF CEO and Co-Founder **Susan L. Solomon** and BBDF Founder **Craig Benson** presented this unique, multi-stakeholder collaboration at the **Partnering For Cures** conference in Manhattan on November 17th.

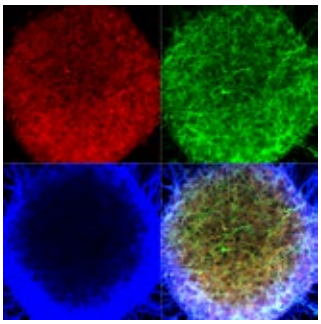
## COLLABORATION TO FIND CLUES TO NEUROPATHY

NYSCF and the **Charcot-Marie-Tooth Association (CMTA)** are working together to research and find cures for neuropathies, a group of genetic and progressive diseases that affect the peripheral nerves, the nerves outside of the brain and spinal cord. To date, there are no known treatments that stop or slow down the progression of Charcot-Marie-Tooth (CMT) diseases and there are no known cures.

The NYSCF Research Institute is making induced pluripotent stem (iPS) cell lines from CMT patients' skin samples. These stem cell lines will be used to make neurons to study the different forms of CMT in a dish, including attempts to correct the specific gene mutations causing the disease.

This research may lead to new treatments and cures for all forms of CMT and, more broadly, a better understanding of all peripheral neuropathies.

# NYSCF LAUNCHING LARGEST EVER STEM CELL REPOSITORY IN 2015



*iPSC-derived midbrain dopaminergic neurons*

The NYSCF Research Institute will make available the largest ever number of stem cell lines to the global scientific research community through the launch of a stem cell repository in 2015. Stem cell research holds incalculable promise to find cures and treatments for a wide variety of devastating diseases, and while many scientists pursue this type of research around the world, they are often hobbled by a lack of access to stem cell lines to adequately conduct their work.

To make these resources available, NYSCF developed a technology to create a large collection of stem cell lines representing the world's population. This unique platform, known as the **NYSCF Global Stem Cell Array™**, is an automated robotic system for stem cell production and is capable of generating cell lines from patients with various diseases and conditions and from all genetic backgrounds.

This resource is crucial to accelerating stem cell research around the world by providing easily searchable and accessible stem cell lines as well as reducing duplication of efforts and facilitating collaboration and partnership.

# SEARCHING FOR THE BEST CELLS FOR REPLACEMENT THERAPIES

It is not yet known which type of cellular reprogramming technique will yield the best cells for the development of new cell therapies for chronic and acute diseases. To better understand this, NYSCF scientists conducted a landmark study comparing stem cells derived using two different methods: induced pluripotent stem (iPS) cells and embryonic stem cells created by somatic cell nuclear transfer (SCNT), a technique pioneered by NYSCF scientists. They found that cells made using the two methods had similar numbers of mutations and determined that both approaches should continue to be pursued in the stem cell field.

*Bjarki Johannesson, PhD, and Dieter Egli, PhD*

The NYSCF Research Institute is one of the only laboratories in the world that currently pursues all forms of stem cell research, including SCNT and iPS cell techniques, for creating stem cells. The NYSCF Research Institute was established in 2006 in part to provide a safe haven to conduct SCNT research. NYSCF Senior Research Fellow and NYSCF – Robertson Investigator **Dr. Dieter Egli** was a corresponding author and NYSCF – Druckenmiller Postdoctoral Fellow **Dr. Bjarki Johannesson** was first author on the study published in *Cell Stem Cell* on November 6th.

**SAVE THE DATE**

**TENTH ANNUAL NYSCF TRANSLATIONAL STEM CELL RESEARCH CONFERENCE**

OCTOBER 28 - 29, 2015
THE ROCKEFELLER UNIVERSITY

# LEADING STEM CELL SCIENTISTS CONVENE AT NYSCF'S NINTH ANNUAL CONFERENCE

Leaders in translational stem cell research from around the world gathered at NYSCF's Ninth Annual Translational Stem Cell Research Conference, held on October 22nd and 23rd at The Rockefeller University in Manhattan.


*From left to right: Panelists of "Large Scale, Big Data" Feng Zhang, PhD, Eric Schadt, PhD, Scott Noggle, PhD, John Greally, PhD, and Susan. L. Solomon, JD*

Highlights included a panel discussion, "Large Scale, Big Data: Stem Cell Innovation," chaired by NYSCF CEO **Susan L. Solomon**, **JD**, featuring **John Greally**, **PhD**, Albert Einstein College of Medicine, **Scott Noggle**, **PhD**, The NYSCF Research Institute, **Feng Zhang**, **PhD**, Broad Institute of MIT and Harvard and Massachusetts Institute of Technology, and **Eric Schadt**, **PhD**, the Icahn School of Medicine at Mount Sinai. Presentation highlights included talks by **Michael Milone**, **MD**, **PhD**, University of Pennsylvania, on a promising new cancer therapy currently in clinical trials and by **Chuck Murray**, **MD**, **PhD**, University of Washington, on his latest work using stem cells for heart regeneration.

**Sir Ian Wilmut**, **FRS**, **FRSE**, of the University of Edinburgh, made a "call to action" for a global stem cell haplobank in his keynote address at the conclusion of the first day. **Rudolf Jaenisch**, **MD**, of The Whitehead Institute, gave the second day's keynote address on the exciting developments over the past few years in using stem cells and genome engineering technologies in the development of models of diseases such as Parkinson's disease and Rett syndrome.

## NYSCF INNOVATORS: THE LATEST BREAKTHROUGHS

NYSCF – Robertson Neuroscience Investigator

**Ed Boyden**, **PhD**, MIT Media Lab, created a modified bacteria that responds to red wavelengths of light, allowing researchers to silence neural activity in the brains of awake mice when the bacteria is activated. This research, published in *Nature Neuroscience*, could pave the way for use in humans to treat **epilepsy** and other neurological disorders.

NYSCF – Robertson Stem Cell Investigator

**Gabsang Lee**, **PhD**, Johns Hopkins University School of Medicine, successfully reprogrammed patients' skin cells directly into neural crest cells. This research, published in *Cell Stem Cell,* has the potential to transform and accelerate research on all neural crest disorders such as **familial dysautonomia** among others.

NYSCF – Robertson Stem Cell Investigator

**Kristen Brennand**, **PhD**, Icahn School of Medicine at Mount Sinai, showed that human neural cell models of **schizophrenia** secrete increased amounts of three types of neurotransmitters commonly associated with many psychiatric disorders. This research, published in *Stem Cell Reports*, could help identify a chemical basis for schizophrenia.

NYSCF – Robertson Stem Cell Investigator

**Derrick Rossi**, **PhD**, Harvard University, used a gene editing technology called CRISPR-Cas to modify human blood-forming stem cells to block **HIV** from invading and destroying the immune system. The research, published in *Cell Stem Cell*, could lead to new treatments and cures for HIV.

NYSCF – Robertson Stem Cell Investigator

**Paola Arlotta**, **PhD**, Harvard University, discovered the first co-regulator gene, or one gene that is able to regulate the expression of large numbers of genes. The research, published in *Nature Neuroscience*, related to motor neurons - the neurons responsible for fine motor function affected in **ALS**.

NYSCF – Robertson Stem Cell Investigator

**Alex Meissner**, **PhD**, Harvard University, performed cost-benefit analysis on genome-wide approaches for epigenetic analysis and concluded that bisulfate sequencing was the best method. These results, published in *Nature Methods*, will allow improved characterization of stem cell populations.

NYSCF – Druckenmiller Fellow

**Raffaella Di Micco**, **PhD**, New York University School of Medicine, identified a key protein, implicated in many cancers, that appears to play a pivotal role in keeping stem cells immature. Published in *Cell Reports*, this finding has large implications on **cancer** research and treatments.

**SAVE THE DATE**

## THE TENTH ANNIVERSARY NYSCF GALA AND SCIENCE FAIR

OCTOBER 20, 2015

# NYSCF'S NINTH ANNUAL GALA AND SCIENCE FAIR SALUTES STEM CELL HEROES

*The Ninth Annual NYSCF Gala and Science Fair. Clockwise from top left: 2014 NYSCF Stem Cell Heroes* ***Rich Rundle****,* ***Sabrina Bertucci****,* ***Anne Mai****, and* ***Vincent Mai****;* ***Chuck Close*** *and* ***Dorothy Lichtenstein****;* ***Barbara Stovall Smith*** *and* ***Susan L. Solomon****;* ***YaYa Cantu****,* ***Danny Freytes, PhD****,* ***Sandra Lloyd****,* ***Giuseppe Maria De Peppo, PhD****, and* ***Gregory Lallos****;* ***Kim Charlton****,* ***Stephen Meringoff****, and* ***Charlotte Meringoff****;* ***Richard Parsons****;* ***Nancy*** *and* ***Fred Poses****;* ***Richard Massey, PhD****, and* ***Tarra Bandet****;* ***Karen Finerman*** *and* ***Lawrence Golub****.*

# 2014 NYSCF - ROBERTSON PRIZE WINNER ADVANCES TREATMENT FOR DEVASTATING SKIN DISEASE

*2014 NYSCF – Robertson Prize winner, Marius Wernig, MD, PhD*

Fourth annual **NYSCF – Robertson Stem Cell Prize** winner **Marius Wernig, MD, PhD**, and team created induced pluripotent stem (iPS) cells from patients with a severe blistering skin disease, **epidermolysis bullosa**, and reprogrammed these cells, correcting the genetic, disease-causing defect. The reprogrammed cells formed normal human skin when grafted into mice. This research, published in *Science Translational Medicine* on November 26th, marks a major advance against this painful and disfiguring genetic disease and lays the foundation for a definitive cell replacement cure.

Dr. Wernig is an Associate Professor in the Institute for Stem Cell Biology and Regenerative Medicine and the Department of Pathology at Stanford University School of Medicine where he focuses on using iPS cells and induced neuronal cells for disease modeling and as a potential cellular therapy.

Dr. Wernig is a member of the inaugural 2010 class of NYSCF – Robertson Stem Cell Investigators and is the first NYSCF – Robertson Investigator to receive the NYSCF – Robertson Stem Cell Prize. NYSCF – Robertson Prize winners each receive $200,000 and an award sculpture designed by architect, NYSCF Leadership Council member, and 2009 **NYSCF Humanitarian Award** recipient **Frank Gehry**.

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# NYSCFNEWSupdate

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## 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 research that is 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.

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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:** Supporting and enabling the unrestricted pursuit of the most advanced stem cell research, both in our own laboratory and through collaborations with major medical research institutions.
- **NYSCF Fellowship and Investigator Programs:** Supporting and training the next generation of scientists, both at the postdoctoral level as Fellows, and as Investigators pursuing innovative work that translates research into cures.
- **NYSCF Conference and Symposia:** Convening the preeminent annual translational stem cell research conference and an on-going series of programs for scientists, policymakers and the public.
- **NYSCF – Robertson Stem Cell Prize:** Honoring the most significant achievement in stem cell research each year.