



2015 Stem Cell Heroes Jeff Gernsheimer and Jack Gernsheimer

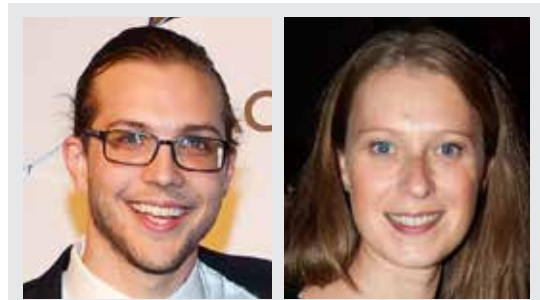
“NYSCF GIVES US HOPE”

Patients are the lifeblood of NYSCF’s research and central to all of its activities. Without their participation, NYSCF’s work would not be possible.

Some of NYSCF’s most important advocates are its research participants. **Jeff and Jack Gernsheimer** are identical twins, though only Jack suffers from Parkinson’s disease. Jack and Jeff contributed skin biopsies for research, which led to an important discovery that may lead to better treatments and new therapeutic approaches for all Parkinson’s patients. “NYSCF is a symbol of hope. Despite the gravity of the diagnosis, there’s a lot of hope for a cure,” Jack said. Jack and Jeff are just two examples of the thousands of individuals who have been critical participants in NYSCF’s research.

NYSCF’s scientists are also affected by and committed to research on the frontlines of patient treatment. NYSCF researcher **Daniel Browne** is a diabetes advocate and type 1 diabetic. Browne joined NYSCF in 2015 to take his disease into his own hands by becoming a NYSCF diabetes researcher. Similarly, NYSCF Principal Investigator **Dr. Valentina Fossati** leads a research team to find new treatments for multiple sclerosis, a disease she was diagnosed with six years ago. Her research discoveries include the development of an accelerated protocol to make the cells affected in MS, an important breakthrough that will fast-track all current stem cell research on the disease.

Patients are NYSCF’s greatest allies and their support and engagement allow NYSCF to move forward and accelerate cures for the devastating diseases of our time.

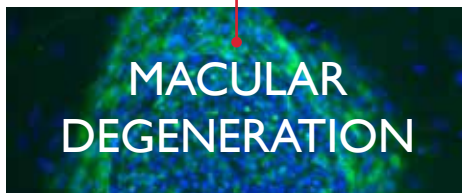


NYSCF Researcher Daniel Browne and NYSCF Investigator Dr. Valentina Fossati

CLINICAL TRIALS BEGIN!

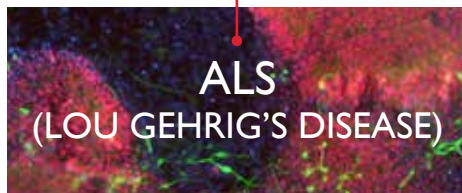
NYSCF creates a global network of preeminent researchers who use NYSCF technology and support to translate their research into new treatments and cures. Less than a decade since scientists first generated patient-specific stem cells in 2007, patients now reap the benefits of NYSCF-supported pioneering research. Today, NYSCF-supported scientists are starting clinical trails to enable regenerative medicine, developing new disease prevention strategies, and bringing new cures to the clinic.

NYSCF Research Institute



MACULAR DEGENERATION

Inaugural NYSCF–Robertson Stem Cell Prize recipient Pete Coffey, PhD, co-leads The London Project to Cure Blindness, which, with NYSCF support treated its first patient for ‘wet’ age-related macular degeneration with a stem cell transplant.



ALS (LOU GEHRIG’S DISEASE)

NYSCF-supported research at Harvard University modeling ALS, Lou Gehrig’s Disease, in stem cells discovered a drug on the market for epilepsy may be protective of motor neurons, the cells affected in ALS. Patients are now being recruited for clinical trials to test the efficacy of this treatment.



MITOCHONDRIAL REPLACEMENT

NYSCF developed a technique to prevent the mother-to-child transmission of mitochondrial diseases, rare but often-fatal diseases. Similar to in-vitro fertilization, this technique was approved by the United Kingdom for clinical trials.

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Dear Friends,

This has been an extraordinary year for NYSCF. We celebrated our tenth anniversary, which means that we can now look back on a full decade of truly path-breaking scientific discoveries enabled by private philanthropy. But we cannot afford to look back for long; NYSCF's future is calling us to keep on moving forward.

We are very excited to announce that we are beginning construction on a brand new laboratory on West 54th Street in Manhattan, which will become the new home of the expanded NYSCF Research Institute late in 2016. Our offices will move there as well, and with all of NYSCF's personnel under the same roof for the first time, we look with excitement toward the synergy that will further accelerate research towards cures.

Because of private philanthropy we have been able to fund the high-risk/high-reward research that is already paying dividends in clinical trials. Three clinical trials based on NYSCF research are underway now, and there are more to come. Private philanthropy has provided us the resources to build pioneering technologies such as the NYSCF Global Stem Cell Array. NYSCF continues to set the pace for what stem cell research can and will do.

NYSCF's generous supporters have truly changed stem cell science, and we are deeply grateful to all of you for all that you have done. Only with your continued help can we deliver on the promise of finding cures through stem cell research. We have come a long way in our first decade, and NYSCF is only beginning. Please help us to reach what we truly believe will be the decade of cures.

Warmest wishes for a happy and healthy new year,

Susan L. Solomon
 CEO and Co-Founder



At NYSCF's 10th Anniversary Gala, attendees learned from NYSCF researchers firsthand at Science Fair stations



The NYSCF Global Stem Cell Array automates the production of hundreds of stem cells from patients in tandem

NYSCF'S 10TH ANNIVERSARY GALA CELEBRATION

NYSCF honored 2015 Stem Cell Heroes Mark McCauley, Jack Gernsheimer, Jeff Gernsheimer, and recipient of the 2015 NYSCF Leadership Award, Stephen M. Ross



Top row from left: Joan Hornig, Chuck Close, and Eve Xanthopoulos; Shirley Cook and David Alan Grier; Mark McCauley, Edith Lipper, and Kyle McCauley

Middle row from left: Jay Eastman, Shelly Lazarus, John Eastman, and Jodie Eastman; David Rockwell, Roy Furman, Susan L. Solomon, Stephen M. Ross, and Kara Ross; Dorian Goldman and Marlene Hess

Bottom row from left: Alan Fisher and Ronne Fisher; Dorothy Lichtenstein and Karen E. Burke, MD, PhD; Jack Gernsheimer, Carmen Dusenberry, and Jeff Gernsheimer

2015 NYSCF – ROBERTSON PRIZE WINNER LEADS A REVOLUTION IN CANCER TREATMENT

Dr. Franziska Michor was awarded the 2015 NYSCF – Robertson Stem Cell Prize for her groundbreaking work applying mathematical models to understand the evolution of cancer biology. Using complex quantitative techniques, Dr. Michor's research calls into question current drug regimens for cancer treatments. Based on her research, new strategies introducing different drugs at specific time intervals are currently in clinical trials to optimize treatment for non-small-cell lung cancer and pro-neural glioblastoma, a type of brain tumor.

Dr. Michor is a Professor of Computational Biology at the Dana-Farber Cancer Institute and in the Department of Biostatistics at the Harvard T.H. Chan School of Public Health. With NYSCF support, Dr. Michor intends to expand her work to study new types of cancer and potential treatments.



Dr. Franziska Michor receives NYSCF – Robertson Stem Cell Prize designed by celebrated architect Frank Gehry

THE NYSCF RESEARCH INSTITUTE: AUTOMATING UNDERSTANDING PRECISION MEDICINE

Precision medicine refers to treatments tailored to individual patients' DNA, lifestyles, and disease prognosis. Precision medicine pushes clinicians to think beyond the one-size-fits-all treatments that have predominated in medicine.

How NYSCF accelerates precision medicine:

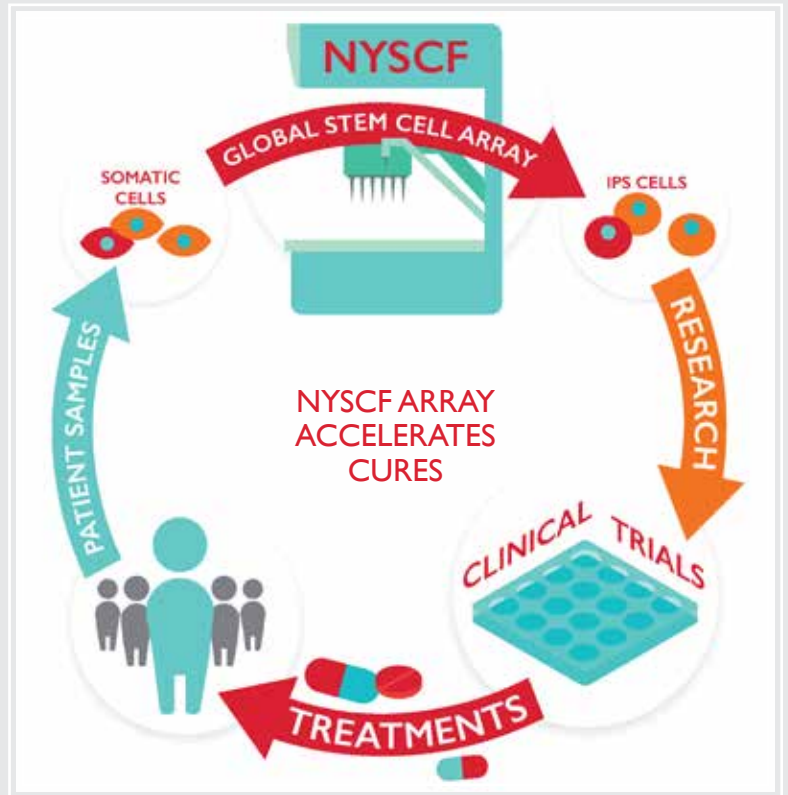
Major biotechnology breakthroughs like the NYSCF Global Stem Cell Array accelerate precision medicine by automating the production of stem cells from hundreds of patients' cells to study how disease actually manifests in patients, and provide the necessary resources to accelerate patient-specific care.

How Array-created stem cells are used:

Currently, stem cells are used to study diseases in patient cells, screen for potential new treatments, and trial treatments before testing on patients. NYSCF is working to create stem cell transplants to treat patients with their own cells, synergizing regenerative and precision medicine.

Why massively parallel research is important:

Massively parallel refers to technologies that produce large-scale results in tandem. The NYSCF Array generates hundreds of stem cells at the same time, while computing detailed information about the cells – freeing researchers to focus on cures.



PARTNERSHIPS TO ACCELERATE CURES

NYSCF's pioneering partnerships and groundbreaking research collaborations empower NYSCF to push the stem cell research field towards realizing the promise of precision medicine. These three new collaborations will join the over 50 leading global institutions NYSCF already works closely with to bring new treatments and cures to patients. Teaming up with local and international organizations enables NYSCF to develop new capabilities, technologies, and techniques, promoting life-saving treatments around the world.



NYSCF and Q-State Biosciences, a biotechnology company that developed imaging technology to probe the electrical properties of cells, partnered this fall to automate the production of stem cells from patients' blood samples for the first time using the one-of-a-kind NYSCF Global Stem Cell Array technology.



NYSCF automates the creation of high-quality stem cell lines from umbilical cord blood tissue in collaboration with Cord Blood Registry (CBR). Using the NYSCF Global Stem Cell Array, NYSCF generates iPS cells from umbilical cord tissues from healthy newborns, collected and banked after birth by CBR.



NYSCF signed a memorandum of understanding with Korea Health Industry Development Institute (KHIDI) of the Republic of Korea, setting the stage for and promoting future international collaborations in the areas of health care and medical science as they relate to stem cells research.

THE FUTURE OF PERSONALIZED MEDICINE

MAKING MEDICINE PERSONAL

NYSCF scientists published a paper in *Nature Methods* describing the successful design of a revolutionary, high-throughput, robotic platform that automates and standardizes the process of transforming patient samples into stem cells. This unique platform, the NYSCF Global Stem Cell Array, for the first time gives researchers the scale to look at diverse populations to better understand the underlying causes of disease and create new individually tailored treatments, enabling precision medicine in patient care.

Researchers outside of NYSCF's Research Institute need to work by hand to create pluripotent stem cells from adult cells, a time consuming and laborious process that produces highly variable results. Elevating stem cell production from a handmade process to automation allows NYSCF to work at an unparalleled scale and to merge genomic data with genetically diverse stem cell populations to study disease onset and outcome.



The NYSCF Global Stem Cell Array gives NYSCF researchers the power to understand patients' cells

“For many common diseases such as diabetes and Alzheimer’s, using stem cell models to understand these diseases has relied on a few rare genetic mutations affecting only a small percentage of all sufferers. Therefore, it has been difficult to study the more common genetic risk factors that affect the majority of patients with these diseases using stem cell models. Our automated system will enable large-scale stem cell experiments needed to understand how these risk factors directly contribute to disease,” **Dr. Scott Noggle**, NYSCF Vice President of Stem Cell Research and senior author of NYSCF’s paper published in *Nature Methods*, explained.

The paper describes how the NYSCF Array reduces the variability that often obscures disease and developmental patterns between stem cells enabling researchers to work towards treatments for patients and providing a platform to conduct ‘clinical trials in a dish,’ giving researchers the power to anticipate patients’ responses to drugs before introducing treatments into humans.

NOBEL LAUREATE SOUNDS CRITICAL ALARM



Nobel Laureate Sir John Gurdon speaks at The NYSCF Conference

Nobel Laureate Sir John Gurdon, University of Cambridge, gave an impassioned talk at The NYSCF Conference, the 10th annual conference for translational stem cell research, on the need to include patients in larger conversations surrounding treatment. Dr. Gurdon advocated for patients’ involvement in discussions on testing requirements and on what restrictions regulators should impose before approving stem cell therapies. He implored patients to engage in the debate that is currently dominated by lawyers and other stakeholders.

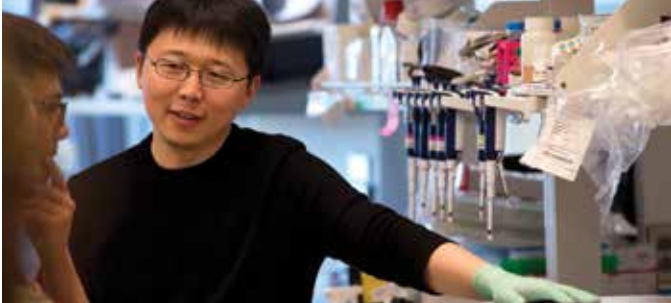
Patients have the most to gain and the most to lose in the critical decisions that guide the leap from laboratory testing to therapies in people. Reiterating the foundations of NYSCF research, Dr. Gurdon’s call reinforced the need to involve those directly affected by new treatments, patients and their loved ones, in

these complicated healthcare questions. As the group that has the most invested in cutting-edge research, it does patients a disservice to rely solely on regulatory bodies to move forward with life-changing therapies.

NYSCF Investigator Dr. Dieter Egli pioneered techniques that will prevent the inheritance of rare and fatal mitochondrial diseases. NYSCF and Dr. Egli now await approval for clinical trials of this technique in the United States. “Breakthroughs in the laboratory are being made continuously, but progress to the clinic requires the call of the patients who need it,” he remarked. “Though ensuring safety and efficacy through regulatory panels is important, going forward to the clinic will always remain a greater risk than performing another research study. To advance the most promising treatments, the patient’s voice is most effective.”

NYSCF INNOVATOR UPDATES

ENGINEERING DNA TO CURE DISEASES



Dr. Feng Zhang teaches in his Lab

(Photo: Len Rubenstein courtesy of Broad Institute Communications)

NYSCF – Robertson Investigator **Dr. Feng Zhang** has spurred a revolution in research with his discovery of a system that scientists can use to edit DNA, CRISPR-Cas9. CRISPR technology, featured alongside Dr. Zhang in *The New York Times* and *The New Yorker* in November, has accelerated scientists’ understanding of genes and made the study and modeling of disease incredibly simple.

Reported in his December *Science* paper, Dr. Zhang’s lab engineered the editing protein, Cas, to increase its specificity, enabling it to only cut DNA sections of interest. Expanding all researchers repertoire of tools to understand and harness natural processes, CRISPR gene editing systems are also regarded for their therapeutic potential.

CONTROLLING NEURONS WITH LIGHT



Dr. Ed Boyden



Mark Zuckerberg and Yuri Milner

NYSCF – Robertson Neuroscience Investigator **Dr. Ed Boyden** received a Breakthrough Prize. Internet pioneers including Sergey Brin of Google, Mark Zuckerberg of Facebook, Yuri Milner of DST Global, and Jack Ma of Alibaba, established The Breakthrough Prize to bring celebrity status to scientists to inspire the next generation to enter the STEM pipeline.

Dr. Boyden earned the recognition for his seminal discoveries in neuroscience. He created optogenetics which allows researchers to control neurons with light, extending neuroscientists’ capacity to understand the brain.

2015 NYSCF – ROBERTSON INVESTIGATORS

This year’s five new **NYSCF – Robertson Investigators**, together with the 31 Investigators, joined the 52 **NYSCF – Druckemiller Postdoctoral Fellows** and the 45 scientists at the NYSCF Research Institute to form NYSCF’s scientific community: a network of over 130 top scientists at leading institutions worldwide.

Each NYSCF – Robertson Investigator receives 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.

NEUROSCIENCE INVESTIGATORS



Hillel Adesnik, PhD

University of California, Berkeley

Dr. Adesnik focuses on revealing the neural basis of sensory perception at the synaptic, systems, and behavioral levels.



Dragana Rogulja, PhD

Harvard Medical School

Dr. Rogulja studies the genetics of sleep regulation and the molecular regulatory mechanisms and neuronal circuitry humans use to filter environmental stimuli during various stages of sleep.

STEM CELL INVESTIGATORS



Mitchell Guttman, PhD

California Institute of Technology

Dr. Guttman aims to understand how large RNAs that do not serve as a template for protein production can regulate specific sets of genes and the unique role of these RNAs, in controlling cell state decisions in embryonic stem cells.



Justin Ichida, PhD

University of Southern California

Dr. Ichida researches patient-specific disease modeling, next-generation sequencing, and chemical screening to identify new treatments for Lou Gehrig’s Disease and sensorineural hearing loss.



Kristy Red-Horse, PhD

Stanford University

Dr. Red-Horse investigates how cardiovascular stem and progenitors cells behave in three dimensions and at the single cell level, bringing a high-resolution understanding of embryonic development to injury and disease models.

BE PART OF NYSCF'S GROUNDBREAKING NEW HOME!



NYSCF Research Institute and office merge under one roof

Fueled by private philanthropy, NYSCF has achieved extraordinary breakthroughs time and again over the last decade. Launched in 2006 as a “safe haven” laboratory to allow for the most promising stem cell research that the government would not support, what began as a 500 sq. ft. room has quickly evolved into one of the largest independent research institutes in the world. As NYSCF’s research achievements have escalated and its scientific team has expanded, the need for additional space and state-of-the-art facilities to match the growing potential of NYSCF’s research capabilities has likewise grown.

To address this urgent need, NYSCF will relocate to a new state-of-the-art home over the next year. Consolidating its laboratories and administrative offices, the new headquarters will be centrally located in midtown Manhattan and bring NYSCF’s capacities together under one

roof. The new home will allow NYSCF to grow both physically and intellectually, making room for new collaborations and cutting-edge research as well as developing and strengthening New York City as a major biotechnology hub.

With your additional support over the coming months to optimally equip this new home, NYSCF will be positioned to advance its mission of accelerating stem cell research toward cures for the diseases facing patients around the globe today and tomorrow.

ADVANCING THE NYSCF MISSION

Dr. Michael Yaffe joins NYSCF as the Vice President of Scientific Programs. Prior to joining NYSCF, Dr. Yaffe served as the Associate-Director of Research Activities at the preeminent California Institute for Regenerative Medicine. Read more on how he learned about NYSCF and what he is most excited about on the journey towards cures for the patients affected by the major diseases of our time.



NYSCF VP of Scientific Programs, Dr. Michael Yaffe

What led you to want to join NYSCF?

I had observed NYSCF from afar for a number of years and was tremendously impressed by its accomplishments including critical contributions to the study of neurodegenerative diseases, pioneering work utilizing somatic cell nuclear transfer (SCNT) and transformative advances in stem cell technology such as development of an automated stem cell production system, the NYSCF Global Stem Cell Array. I was also attracted by the diversity and comprehensiveness of NYSCF’s activities: support of young stem cell scientists throughout the world, targeted research at the NYSCF lab focused on critical bottlenecks, education in stem cell science directed at both scientists and the broader public through conferences and other programs, and a persistent voice for patient advocacy. I wanted to participate in these activities and contribute to their success.

What excites you the most about the current state of stem cell research?

I am most excited about the novel therapies and new insights into disease that are emerging as a direct result of recent advances in stem cell research. We now have incredibly powerful techniques to derive and study diverse human cell types; these cells enable novel and impactful approaches for disease modeling, drug screening and cellular therapy. I know others share my enthusiasm for the profound medical and scientific opportunities that lie before us.

BRINGING STEM CELL RESEARCH TO STUDENTS



NYSCF researcher Brian Campos shows Harlem Children’s Zone students neurons affected by Parkinsons disease created from patients

In honor of World Stem Cell Day on October 14th, NYSCF scientists guided high school students through the NYSCF Research Institute. The visiting students from **Harlem Children’s Zone’s Promise Academies** learned about regenerative medicine and the different technologies NYSCF scientists develop to respond to the challenges of stem cell research.

Through educational outreach programs such as NYSCF’s Science and Technology Education Program (STEP), NYSCF seeks to empower the next generation of students to pursue careers in science, and hopes to inspire students to imagine themselves in careers they may not otherwise have access to.

FUTURE CURES BEGIN WITH YOUR SUPPORT TODAY!

Please make a gift to help NYSCF advance stem cell research toward the clinic.

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www.nyscf.org/donate
or mail a check to:

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



NYSCF NEWS update

NYSCF 10th ANNIVERSARY GALA



NYSCF NEWS update

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