NYSCF Mission

Accelerating cures for the major diseases of our time through stem cell research
NYSCF Programs

NYSCF Research Institute

NYSCF Innovators: Fellows and Investigators

NYSCF Conferences and Symposia
Pipeline of Leading Investigators

- NYSCF - Druckenmiller Fellows – 50
- NYSCF - Robertson Investigators – 35
- NYSCF Research Institute scientists – 45
NYSCF – Robertson Stem Cell Prize

Peter J. Coffey, DPhil
2011

Kazutoshi Takahashi, PhD
2012

Amy J. Wagers, PhD
2013

Marius Wernig, MD, PhD
2014

Franziska Michor, PhD
2015
NYSCF Conference and Symposia

NYSCF’s Annual Translational Stem Cell Research Conference

• Symposium for top stem cell researchers, policy makers and industry
• Brings together stem cell scientists from around the world

October 26-27, 2016
NYSCF Research Institute

- 45 full time NYSCF researchers
- Invested $160M+ in stem cell research
- Leader in developing stem cell technologies and disease modeling
- Proven ability to develop curative technologies
The NYSCF Research Institute has all the following capabilities *under one roof*:

- Use all forms of stem cells
- Create human disease models
- Perform drug discovery
- Proprietary NYSCF Global Stem Cell Array and technologies
- Bioengineers, industrial engineers, computer scientists
Why Do Cures Take So Long?

Academic Institutions

Identify Disease Causes

Publish Research Papers

? Then what?

• Use small collections of cell lines from a narrow group of patients

Pharmaceutical & Biotech Companies

Drug Development

• 13 Years
• $1-4 Billion
• 99% Fail

• Mainly work on large disease markets
• Public companies - risk averse
• Screen on mice and cells unrelated to the disease
• “Wait and See” for stem cells
NYSCF Provides a Bridge to Cures

connecting research to cures and treatments

Academic Institutions can scale their discoveries

Biotech & Pharmaceutical Companies reduces time, cost, and risk
Objectives:

- Reproducible stem cell production
- Parallel derivation & culture at scale
- Quantitative quality control assays
- Reproducible panels of differentiated cells
- Diverse and disease populations

Connect Genotype to phenotype:

- in vitro GWAS
- “Clinical trials in a dish”
Pluripotent stem cells

Skin or blood sample

Defined factor reprogramming

infect fibroblasts with reprogramming viruses

Diseased cells in the body

Turn candidate drug into therapy

Identify candidate drugs

Screen candidate drugs on diseased cells

Candidate drugs

Diseased cells in a dish

Differentiation and selection

Induced Pluripotent Stem Cells (iPS)

Propagation in Culture
Existing Challenges with iPS cells

- Not standardized
- Not diverse
- Not scalable
A New Technology Platform:
The NYSCF Global Stem Cell Array™
The NYSCF Global Stem Cell Array™

- Automates stem cell production to provide the consistency needed for meaningful drug testing and cell therapies
- Represents the global diversity of the world’s population
- Replicate diseases in a dish, using the human cells that are affected by those diseases (not mouse cells)
- Anticipate how different people will respond to drugs before clinical trials
Automated differentiation

Forebrain Neurons

Dopaminergic Neurons

Beta Cells

Cardiac Cells
NYSCF Repository - *iPS Cells WITHOUT restrictions*

Sharing resources with scientists around the world

<table>
<thead>
<tr>
<th>Diagnosed Disease</th>
<th>diabetes mellitus</th>
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<tbody>
<tr>
<td>Subject Age at Diagnosis</td>
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<tr>
<td>Genetic Alteration(s)</td>
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<td>Ethnicity</td>
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<td>Sex</td>
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<th>Part of Collection</th>
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NYSCF Disease Research Areas

- Bone regeneration
- Cancer
- Diabetes / auto-immune diseases
- Heart disease
- Macular degeneration/Retinal disease
- Neural disorders
  - ALS
  - Alzheimer’s disease
  - Parkinson’s disease
  - Multiple sclerosis
  - Neuropsychiatric
Select NYSCF Research Highlights

2011: First personalized embryonic stem cells

2012: Preventative cure for rare diseases affecting children

2013: First-ever personalized bone grafts

2014: Modeling Alzheimer’s disease in the dish

2014: Personalized stem cells from diabetic patients

2014: Accelerated method for making cells affected in multiple sclerosis

2014: New possible treatment for Parkinson’s disease

2016: Made stem cells with one set of genes
## Select Large-Scale Collaborations

<table>
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<tr>
<th>Collaboration</th>
<th>Logo</th>
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<td>Parkinson’s Progression Markers Initiative (MJFF)</td>
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Extensive Institutional Collaborations & Key Relationships (50+)

International Collaborations
Australia
China
Israel
Japan
Korea
Sweden
Switzerland
United Kingdom
NYSCF STEP

Science and Technology Education Program (STEP)
*Inspiring and training the next generation of scientists*

- **NYSCF Academy**
  - Tours of NYSCF laboratory
  - Seminars at local middle and high schools
  - Career development component

- **NYSCF University**
  - 10-week paid summer internship program
  - College students
  - Assigned mentors in the lab
Initiative on Women in Science and Engineering (IWISE)

Creating and promoting actionable strategies to achieve gender equality in science, medicine, and engineering.
The Next Phase - 619 West 54th Street
New Home for NYSCF
New Home for NYSCF
Thank you

• Health Research Alliance
• Program Committee
• Co-host Doris Duke Charitable Foundation
• Iacocca Family Foundation
• Simons Foundation