



The New York Stem Cell Foundation

Health Research Alliance Members Meeting

Susan L. Solomon
March 31, 2016



NYSCF Mission

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



NYSCF Programs



NYSCF Research Institute



NYSCF
Innovators:
Fellows and
Investigator
s



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

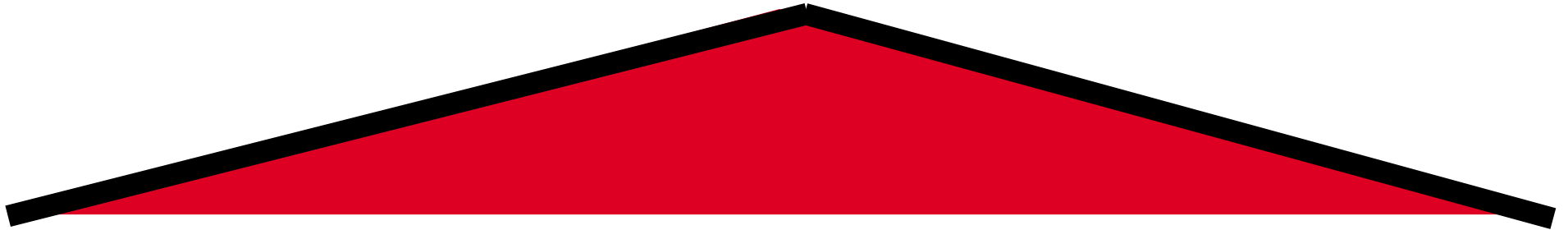


NYSCF Research Institute

- 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



NYSCF: Nonprofit Accelerator



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



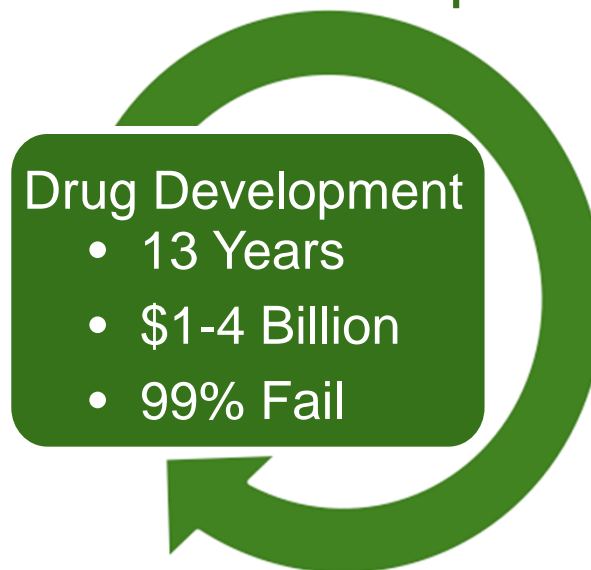
Publish
Research
Papers



?
Then what?

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

Pharmaceutical & Biotech Companies



- 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*

NYSCF Research Institute

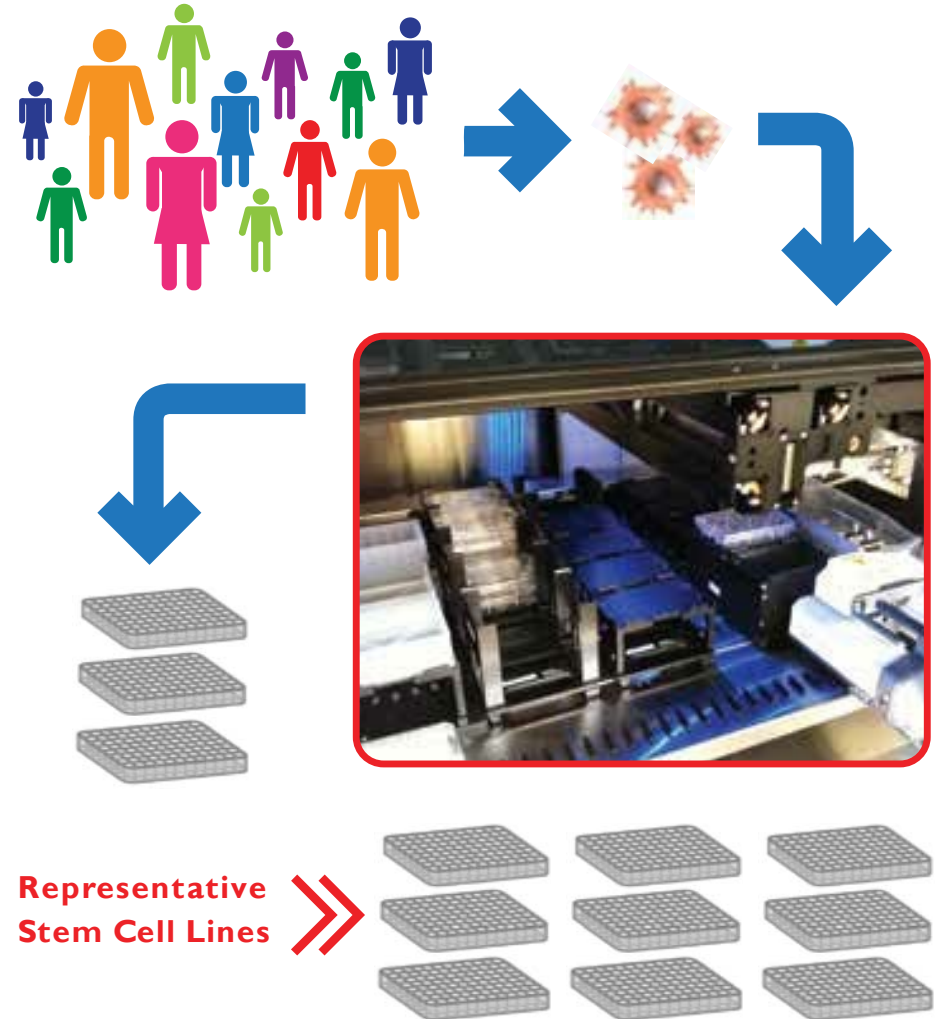
Building infrastructure to industrialize stem cell research

Objectives:

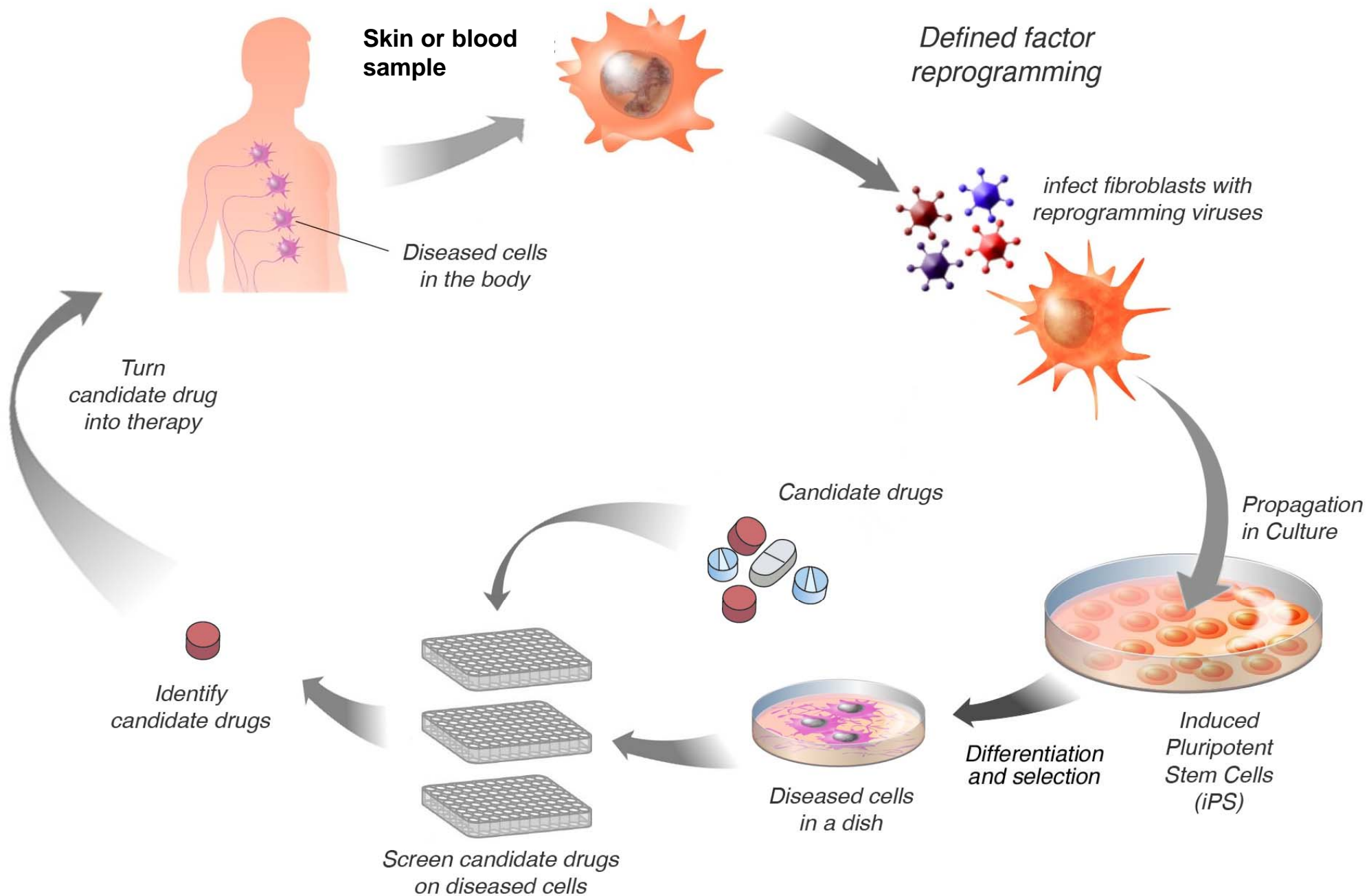
- Reproducible stem cell production
- Parallel derivation & culture at scale
- Quantitative quality control assays
- Reproducible panels of differentiated cells
- Diverse and disease populations

Connect Genotype to phenotype:

- in vitro GWAS
- “Clinical trials in a dish”

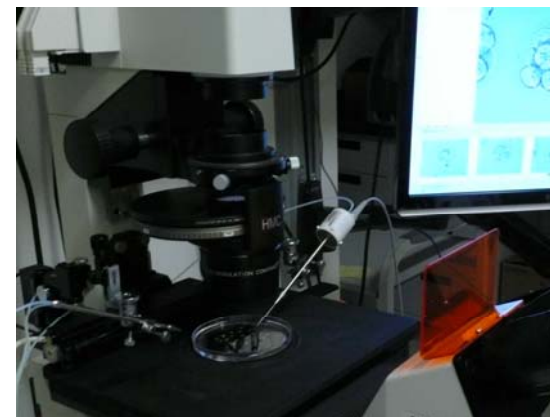


Pluripotent stem cells



Existing Challenges with iPS cells

- Not standardized
- Not diverse
- Not scalable



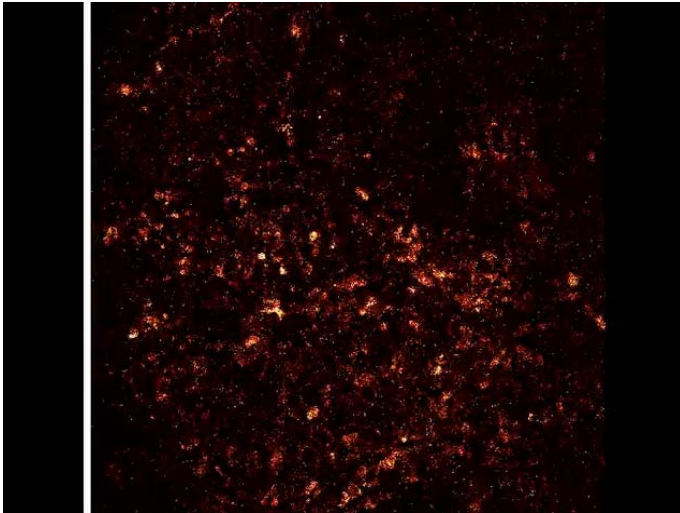
A New Technology Platform: The NYSCF Global Stem Cell Array™

The NYSCF Global Stem Cell Array™

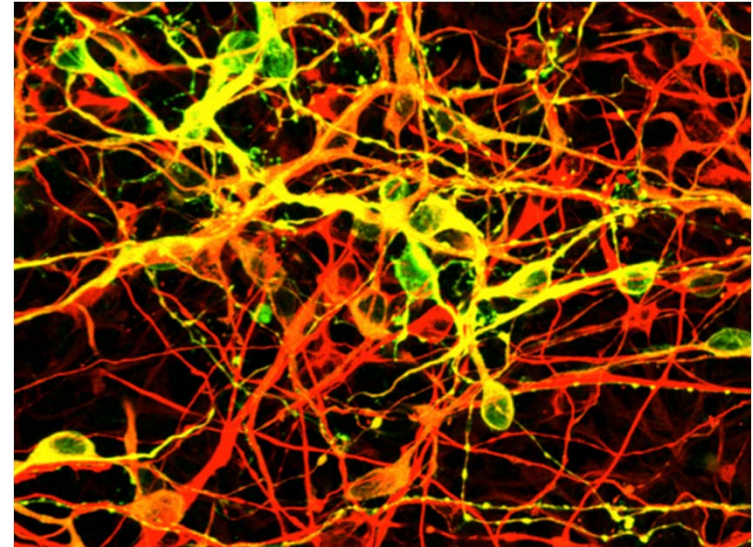
- Automates stem cell production to provide the consistency needed for meaningful drug testing and cell therapies
- Represents the global diversity of the world's population
- Replicate diseases in a dish, using the human cells that are affected by those diseases (not mouse cells)
- Anticipate how different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



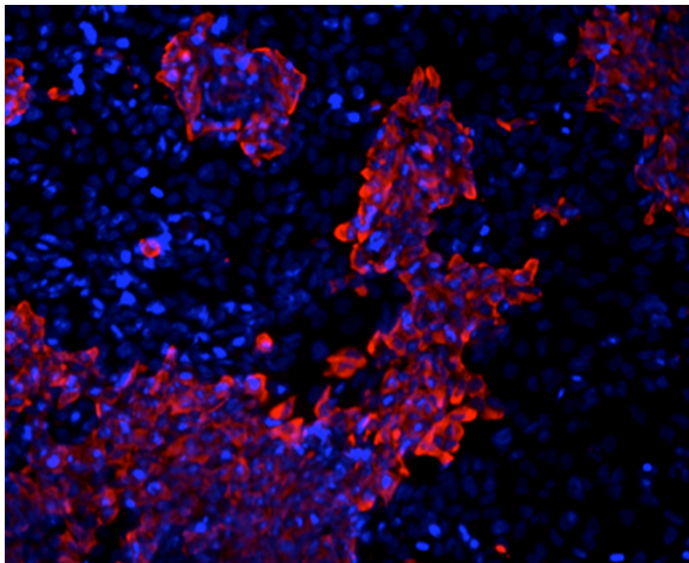
Automated differentiation



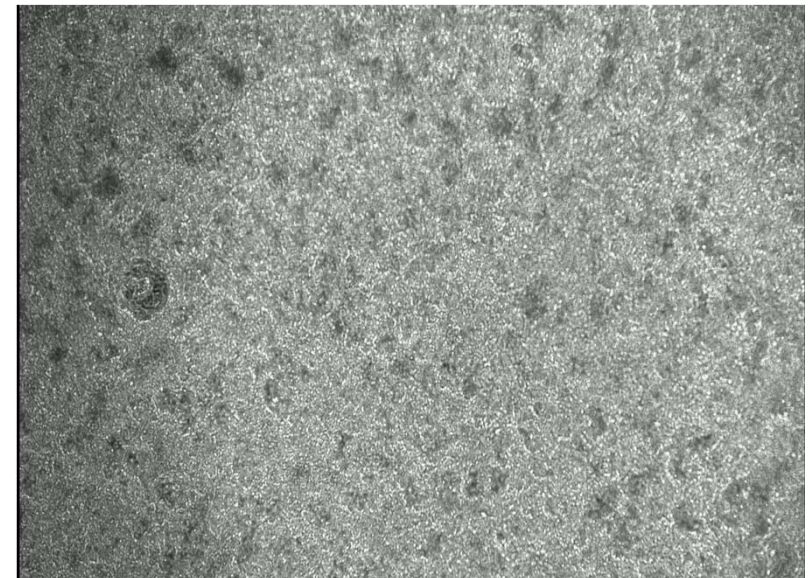
Forebrain Neurons



Dopaminergic Neurons





Beta Cells







Cardiac Cells



NYSCF Repository - *iPS Cells WITHOUT restrictions*



Sharing resources with scientists around the world

Diagnosed Disease  



Subject Age at Diagnosis  



Genetic Alteration(s)  



Ethnicity  

Sex  

AND limit to derived cell lines that meet the following criteria:

Part of Collection  

Induction Method  

Type of QC Performed  

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



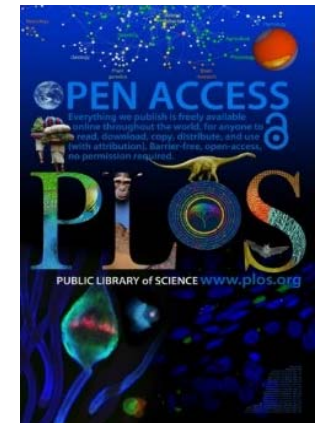
2011: First personalized embryonic stem cells



2012: Preventative cure for rare diseases affecting children



2013: First-ever personalized bone grafts



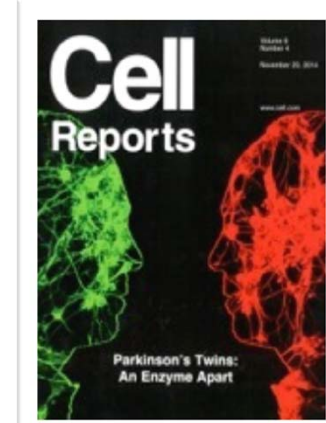
2014: Modeling Alzheimer's disease in the dish



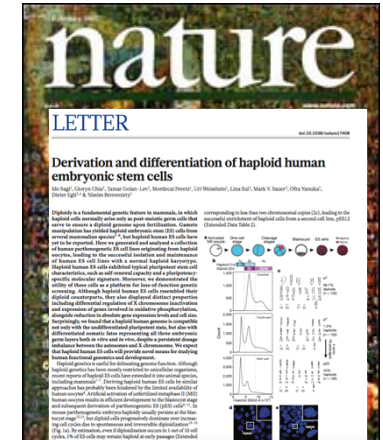
2014: Personalized stem cells from diabetic patients



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



Parkinson's Progression Markers Initiative (MJFF)



PARKINSON'S
PROGRESSION
MARKERS
INITIATIVE

Play a Part in Parkinson's Research

NIH Undiagnosed Disease Program



National Center
for Advancing
Translational Sciences

ORDR
Office of Rare
Diseases Research

Cure Alzheimer's Fund Stem Cell Consortium



Personal Genome
Project

www.personalgenomes.org

Personal Genomes Project

Google



Charcot-Marie Tooth Association



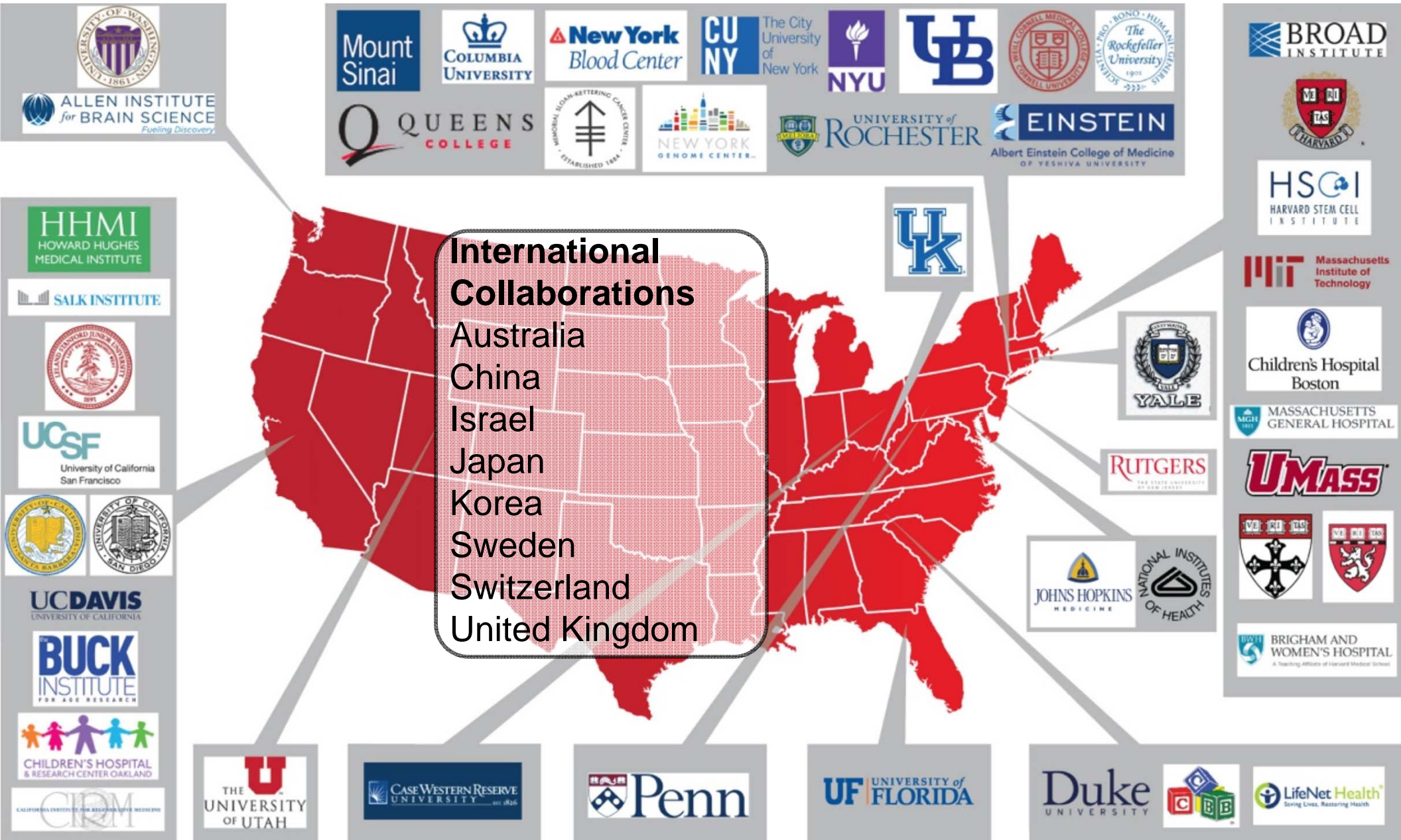
Stanley Center at the Broad Institute



Global Alliance for iPSC Therapies (GAIT)

Haplobank

Extensive Institutional Collaborations & Key Relationships (50+)



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



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