EMORY UNIVERSITY
OFFICE of TECHNOLOGY TRANSFER

Human Stem Cells v3.0

The Holy Grail of Regenerative Medicine

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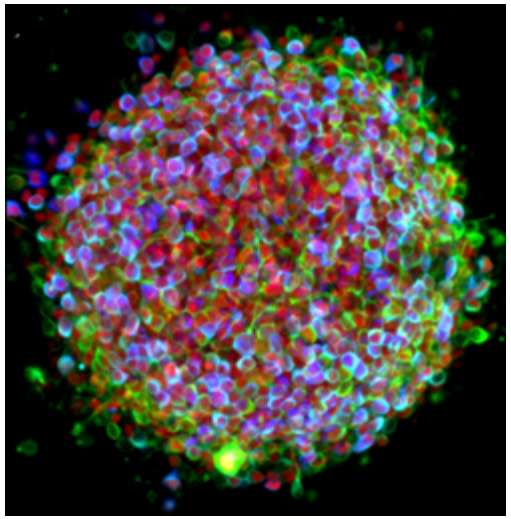

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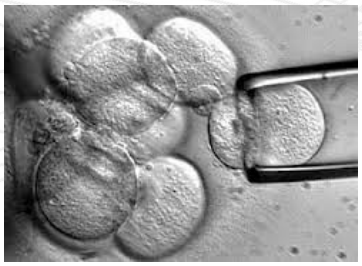
OTT Breakfast Club
December 11th, 2012

Stem Cell Unrealized Promises

- **v1.0** - Embryonic Stem (ES) Cells
  - 1981 mouse ES cells first isolated
  - 1998 human ES cells first isolated



- **v2.0** - Induced Pluripotent Stem Cells (iPS Cells)
  - 1962 frog nuclear transfer experiments
    - Led to Dolly (sheep) cloning
  - 2006 mouse somatic cell reprograming
    - Viral expression vectors to modify gene expression
  - 2012 Nobel Prize in Physiology and Medicine

Stem Cell Use Issues

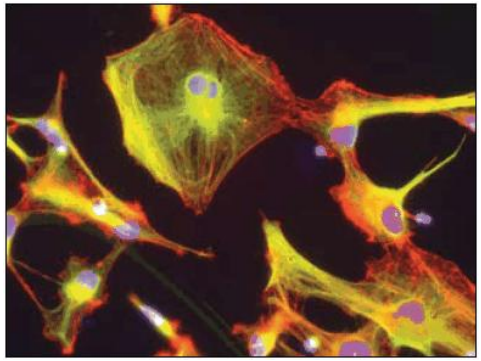
- **v1.0** ES Cells
  - Politics
  - Sourcing
  - Potential Cell/Tissue Rejection

- **v2.0** iPS Cells
  - Potential Cell/Tissue Rejection
  - Exogenous gene insertion and recombination events
  - Potential Cancer Inducing

Fine for Research Tools but Limited Clinical Use

Opportunity - v3.0 Stem Cells

Process that can create iPS Cells using small molecules to reprogram DNA leading to embryonic specific gene expression

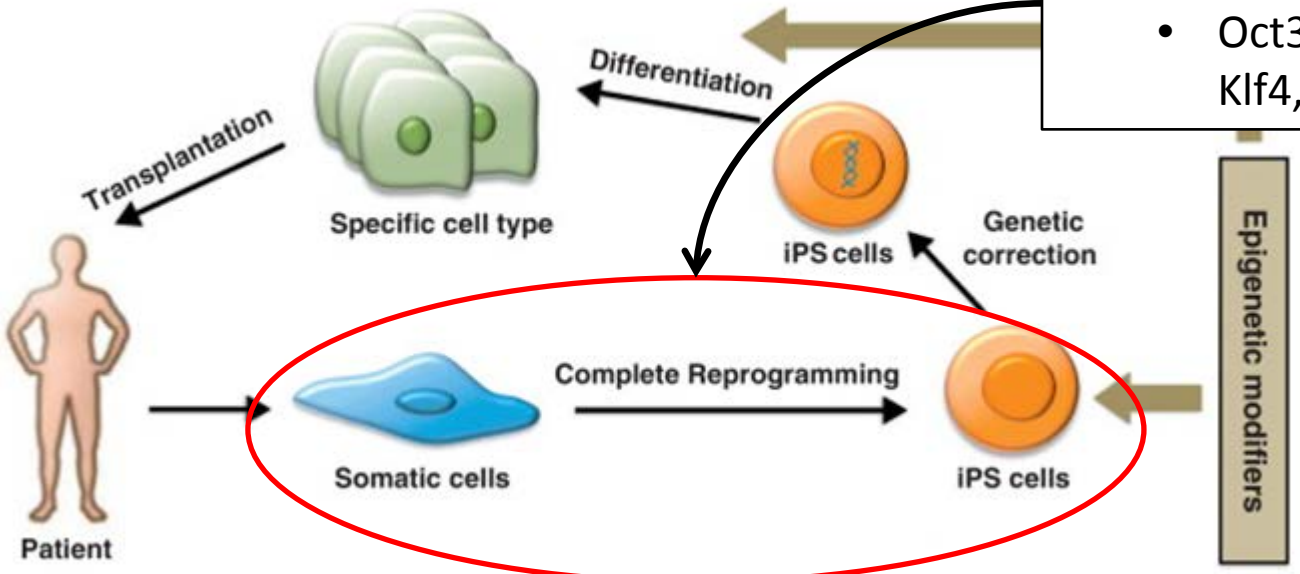


- Autologous
- Viral Expression Vector Free
- Pluripotent Cells
- Allows for Subsequent Cell Differentiation

iPS Cell Generation

v2.0 iPS Cells

- Viral Expression Vectors
  - Oct3/4, Sox2, c-myc (?), Klf4, nanog



v3.0 chemiPS Cells

- Histone methyltransferase inhibitors
- Histone deacetylase inhibitors & valproic acid
- Wint signal modulators

Stem Cell Technology Market

Who's Who - Stem Cell Companies
http://www.stemcellresources.org/who_companies.html
Over 50 companies listed

- **Advanced Cell Technology, Inc.**
- **Applied Tissue Technologies**
- **Cell Genesys**
- **ES Cell International**
- **Neuralstem**
- **Osiris Therapeutics**
- **Stem Cell Innovations, Inc.**
- **Stem Cells Inc.**
- **StemCell Services**
- **StemCell Technologies Inc.**
- **Stem Cell Sciences**
- **Stem Cell Therapeutics**

Ongoing Clinical Trial
- Stargardt Disease
- AMD
- Differentiated retinal epithelial cells derived from hES cells

Ongoing Clinical Trial
- ALS
- Human neuronal stem cell line NSI-566

Ongoing Clinical Trial
- AMD
- Allogenic human neuronal stem cell line

Rapid Technology Integration Expected

Technology Status

- Well Funded Research Program
  - Part of a $25M NSF funded consortium studying stem cells
  - NIH grant support for chemiPS work
  - New compound development with collaborators

- PCT patent application filed – Emory Tech ID 12022
  - 11/08/2012

Technology Summary

Process, with novel small molecules, that can create autologous iPS Cells

	Autologous	Genetically Clean	Easy to Obtain	Regenerative Medicine	Research Tools
chemiPS Cells	Yes	Yes	Yes	Yes	Yes
iPS Cells	Yes	No	Yes	No	Yes
ES Cells	No	?	No	?	Yes

chemiPS Cells Meet All Requirements for Clinical Stem Cell Applications