

### Cells for Therapy and Research

# 26<sup>th</sup> Annual ROTH Conference March 10<sup>th</sup> 2014



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## Forward Looking Statement

This presentation includes certain statements, estimates and projections with respect to the anticipated future business and performance of the Company, which are collectively referred to as forward-looking statements.

Forward-looking statements reflect various assumptions of management that may or may not prove to be correct, and are intended solely to convey our expectations or predictions about the future performance of the Company.

All forward-looking statements are inherently uncertain as they are based on our current expectations and assumptions concerning the future performance of our Company. This is not an offer to sell nor a solicitation to buy any security.



#### Who We Are

### **Stem Cell Regenerative Medicine Company**

#### **Successful Commercial Subsidiaries**

- Biomedical products businesses addressing markets in excess of \$3 B
- \$5.66 M Revenues (TTM)
- Debt free
- Growing 20%+ per quarter (YOY)

#### Stem cell platform targeting therapeutics

- Solves real world problems
- Well protected, large IP estate
- Multiple therapeutic Indications
- Parkinson's program moving to clinical phase within 12 months

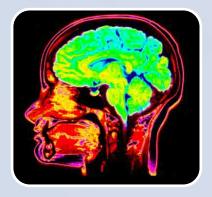


### Core Strengths









# Cutting-edge Science

- Proprietary
- Solves real-world problems
- Unique
- Powerful
- Ethically Superior
- Large Patent Estate

# State of the art Manufacturing

- Research and GMP grade
- Human Cells and Media
- Efficient, Scalable
- Low-cost processes

# Strong Revenue

- 20%+ quarterly revenues growth from subsidiaries
- Excellent Margins
- Synergistic infrastructure

#### Cell Therapy Programs

- Huge commercial opportunities
- Multiple indications
- Strong partnering prospects



# **Key Facts**

Trading Symbol	OTCQB: ISCO	
Corporate Headquarters	Carlsbad, CA	
Stock Price(3/5/14)	\$0.24	
52 Week Range	\$0.13 - \$0.34	
Market Cap. (3/5/14)	\$36.23 M	
Trading Volume (3/5/14)	811,832	
Long-Term Debt	\$0	
Cash on Hand (9/30/13)	\$1,792 K	
Monthly burn (9/30/13)	\$470 K	
Enterprise Value (3/5/14)	\$31.42 M	
Revenue TTM	\$5.66 M	
Full Time Employees	42	
Audit firm	Mayer Hoffman McCann	



#### Commercial Subsidiaries



# Lifeline Cell Technology – a vertically integrated primary human cell research products business

- \$2.3 B Biomedical products market (bcc research 2009)
- Over 130 cells and optimized culture media promits
- Branded and OEM products
  - Growing domestic sales force





# Commercial Subsidiaries (2)



# Lifeline Skin Care - branded anti-aging stem cell skin care products

\$800 M cosmoseutical market (Kline 2011)

**Distribution Channels** 







**E-tailers** 



Physicians
Spa/Med Spa



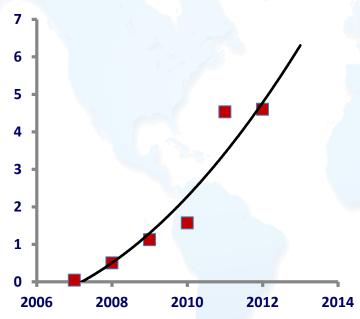
International

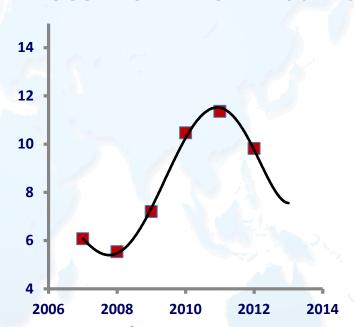
## **Commercial Operations**

#### **Growing revenue diminishing net loss**

Revenue / \$million

Loss from Dev. Activities





Excellent Gross Margins 2012 = 72%

2013 Results to be published next week



## Platform Stem Cell Technology

#### **Cutting-edge Science**

Parthenogenesis. Human pluripotent stem cells from unfertilized eggs

# Clinically superior for allogeneic therapy

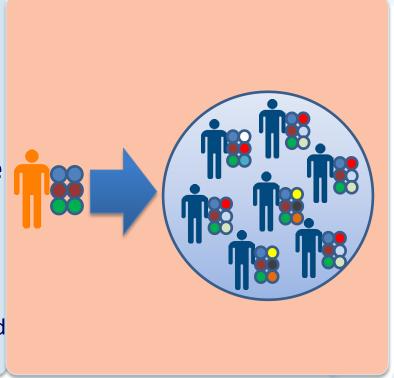
Less rejection (histocompatible )

#### **Pluripotent and Proliferative**

Can be used to treat millions of patients and many diseases

#### **Bypasses ethical concerns**

No viable embryo created or destroyed





## Intellectual Property Investment

Large patent portfolio including stem cell creation and differentiation methods

130 patents and licenses in 14 families

90 patents pending in 5 families

Significant peer-reviewed publications

Nature's Scientific Reports (2013) Cell Stem Cell (2011,2012)



## Manufacturing Capabilities

# Human cell manufacturing capabilities suitable for early-stage clinical trials

- 13,125 sq.ft laboratory including 5 GMP manufacturing suites
- Largest private cryobank of histocompatible human pSC





### R&D Targeting Multiple Diseases

#### Validating the stem cell platform for allogeneic therapy

Program	Target	Pre-Clinical	IND Track	Phase I
Human Neural Stem Cells	Parkinson's disease			
Hepatocyte-like cells	Inherited metabolic liver diseases			
Corneal cell/tissue	Corneal blindness			

#### Success unlocks significant additional value



#### Overview of Parkinson's Disease

#### 2<sup>nd</sup> most common neurodegenerative disease

- 7-10 million people world-wide
- 60k new case / year (Parkinson's disease Foundation)
- \$2.5 billion in sales (2012)

#### **Oral medications**

- Dopamine replacement/ agonists
- Blockers of DA metabolism

#### **Deep brain stimulation**

Surgical implant of neuro-stimulator to modulate brain activity

- Limited to a sub-group of patients
- Unknown long-term safety

#### Fetal tissue implants:

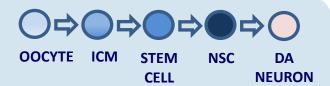
- Ethically controversial
- Immune rejection by host
- Heterogeneity of the grafts
- Graft induced dyskinesia occur in subsets of patients



Disease continues to progress. No cure



## Allogeneic Therapy for Parkinson's



#### **Human Neural Stem Cells (hPNSC)**

Derived from platform technology

#### Self-renewing, multipotent

- Practical, unlimited supply of pure cells
- Not ethically controversial
- May be less immunogenic

#### **Potential mechanism**

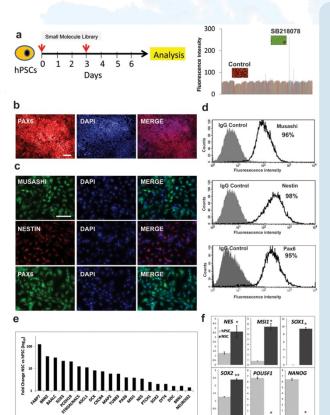
- Neuroprotection: Produce factors that protect against cell death
- Immunomodulation: Migrate to areas of inflammation, inhibit immune response
- Cell Replacement: Differentiate into neuronal cell types lost in PD

#### Value driving - unlocks multiple additional indications

Stroke, Amyotrophic Lateral Sclerosis, TBI



## Manufacturing of hPNSC



#### **Fully characterized**

#### Scalable, low cost GMP protocol

- Suitable for pre-clinical and clinical studies
- Fully characterized for purity, safety and functionality

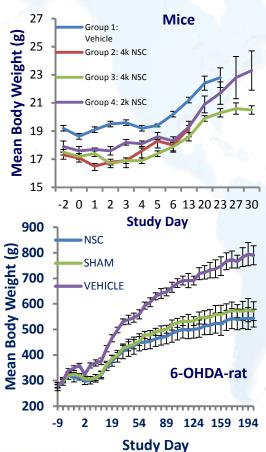
Published in peer-reviewed literature





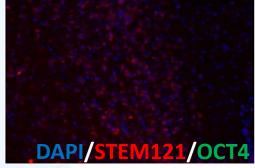
## Good Safety and Tolerability of hPNSC

# Body weight of mice and rats within normal ranges



#### No visible tumors or pluripotent cells





Coronal section of the monkey brain stained with hematoxylin and eosin showing a normal morphology without the presence of ectopic tissue

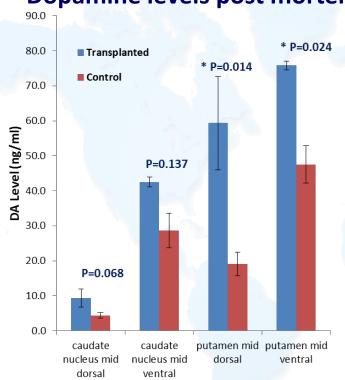
Implantation site stained for human specific antibody STEM121 (in red) and OCT4 (in green), a marker of undifferentiated pluripotent stem cells. OCT4 is completely absent in the graft site.

#### Necropsies revealed all organs to be normal

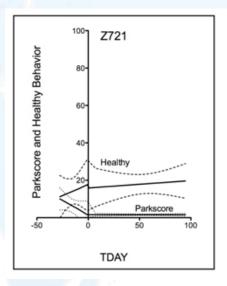
Biodistribution analysis indicated no human cells found in any peripheral organs

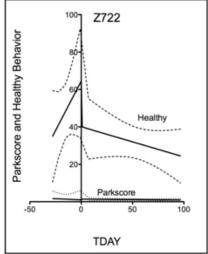
## Primate Proof-of-Concept hPNSC Data

#### Dopamine levels post mortem



# Stable Parkscores and Healthy Behavior for duration of the study





# Functional activity demonstrated in disease model (asymptomatic) primates



## Parkinson's Disease Summary



#### Complete GLP pre-clinical w/ clinical-grade hPNSC

 Primate pharmacology, toxicology, and efficacy Tumorigenicity study in SCID mice

#### Submit IND and begin FIM study w/ Duke

- Single arm, open label, dose escalating study
- < 20 subjects with idiopathic PD, UPDRS < 50</p>
- PI Prof. Mark Stacy, internationally renowned Neurologist with significant clinical trials experience in PD
- Stem cell therapy center of excellence

#### First company using PSC to treat PD



#### World-class Partners

#### Dr. Mark Stacy, MD

Vice Dean for Clinical Research, Neurology at Duke University School of Medicine

#### Dr. Evan Y. Snyder, MD PhD

Chair of the FDA Advisory Committee for Cellular, Tissue and Gene Therapy UCSD and Director of Sanford-Burnham Stem Cell Research Center

#### Dr. D. Eugene Redmond Jr. MD

Professor of Psychiatry and of Neurosurgery, Yale School of Medicine

#### Dr. Jeanne Loring, PhD

Professor of Developmental Neurobiology, The Scripps Research Institute

- Clinical PI
- Neurologist
- Histology
- Data analysis
- Primate Research
- Necropsies
- Genomic analysis
- Characterization



### **Growth Strategy**

#### **Expand Commercial Operations**

- New commercial product lines
  - Additional cell types, new anti-aging products
- New business areas
  - International distribution, Clinical-grade products, OEM products

#### **Near-term therapeutic milestones**

- Parkinson's Disease pre-IND 1Q 2014 COMPLETE
- GLP Pharm/Tox data 4Q 2014 IND soon after
- Parkinson's FIM Phase I/II 2015
- Additional therapeutic indications 2015



### Summary

# Debt free commercial-stage Regenerative Medicine Company

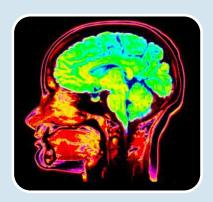
#### **Strong Commercial Operations**

- Significant growth opportunities
- Products already on the market
- \$5.66 M in sales TTM

#### **Proprietary Stem Cell Platform**

- Solves real-world problems
- Initial target Parkinson's disease
- Numerous partnering opportunities
  - Platform and therapy
- Multiple additional indications unlocking huge additional value







Questions?

ISCO.QB

