

## Cells for Therapy and Research

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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.



## RM Company with Substantial Revenues

# Stem cell platform technology targeting high value therapeutic indications:

- First in man study in Parkinson's disease
- Secondary programs in metabolic liver disease and ophthalmology indications
- Potential to unlock multi indications
- Multiple partnering opportunities

# \$5.66 million\* revenue from biomedical products businesses



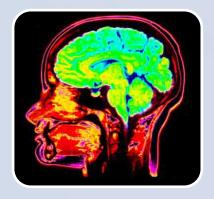


## **Core Company Components**









## Stem Cell Platform

- Unique Benefits
- Differentiated
- Multiple Indications
- Ethically Superior
- Patented and Protected

## Human Cell Manufacturing

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

# Biomedical Products Businesses

- Strong Revenue Growth
- Excellent Margins
- Synergistic Infrastructure

#### Cell Therapy Programs

- Large Unmet Medical Needs
- Neural Stem Cells
- Hepatocyte like Cells
- Extensive Pre-Clinical Datasets



## Platform Stem Cell Technology

**Parthenogenesis**. Human pluripotent stem cells from unfertilized eggs

# Clinically superior for allogeneic therapy

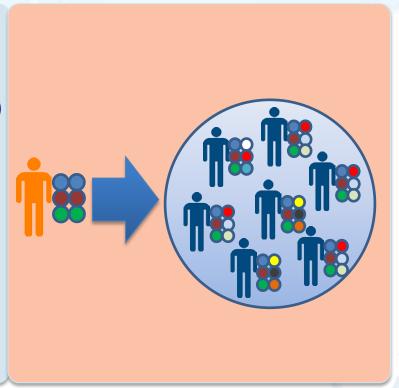
Histocompatible cells (less rejection)

### **Pluripotent**

One cell line can be used to treat millions

### **Bypasses ethical concerns**

No viable embryo created or destroyed





## Intellectual Property Investment

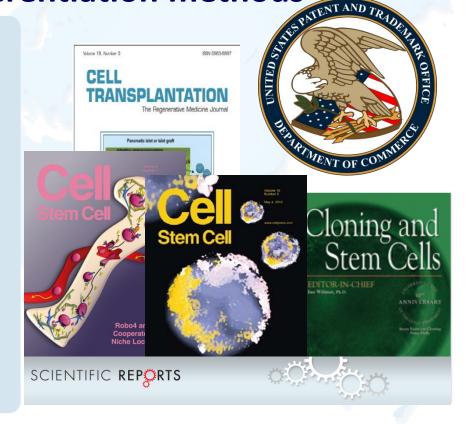
Large patent portfolio including stem cell creation and differentiation methods

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 clinical 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



## hNSC for Allogeneic Therapy

# Self-renewing, multipotent human neural stem cells Derived from parthenogenetic stem cells

- 1. Practical, unlimited supply of pure cells
- 2. Not ethically controversial
- 3. May be less immunogenic

#### **Potential mechanism**

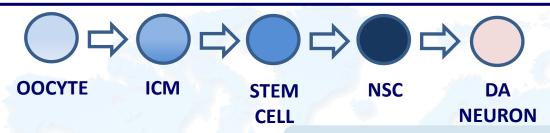
- 1. Neuroprotection: Produce nerve growth factors that protect against cell death
- 2. Immunomodulation: Migrate to areas of inflammation and inhibit immune response
- 3. Cell Replacement: Differentiate into neuronal cell types lost in PD

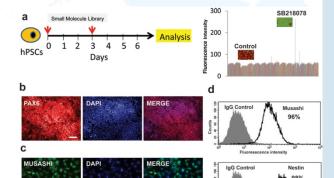
### Value driving - unlocks multiple additional indications

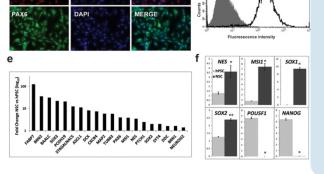
• Stroke, Amyotrophic Lateral Sclerosis, Palsy etc



## hNSC Characterization and Manufacturing







## Scalable, low cost GMP protocol

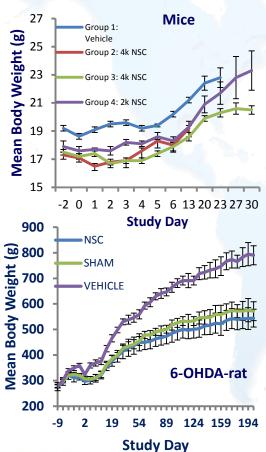
- Suitable for pre-clinical and clinical studies
- Fully characterized for purity, safety and functionality

Published in peer-reviewed literature



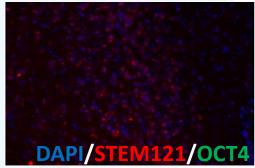
## hNSC Show Good Safety and Tolerability

## 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.

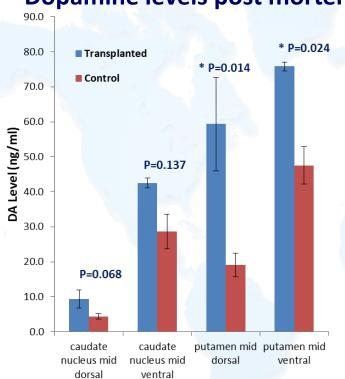
### Necropsies revealed all organs to be normal

Biodistribution analysis indicated no human cells found in any peripheral organs

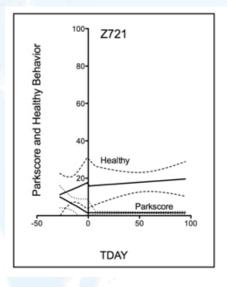


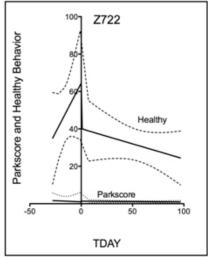
## Primate Proof-of-Concept Data

#### Dopamine levels post mortem



## Stable Parkscores and Healthy Behavior for duration of the study





# Functional activity demonstrated in disease model (asymptomatic) primates



### 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



## Parkinson's Disease 2014 Targets

## **Complete pre-clinical**

- Primate pharmacology, toxicology, and efficacy Mid 2014
- Tumorigenicity study in SCID mice Mid 2014



## Submit IND and begin FIM study w/ Duke

- Single arm, open label, dose escalating study
- < 20 subjects with idiopathic PD, UPDRS < 50</li>
- 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





### **Commercial Subsidiaries**

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## Corporate Structure



Core stem cell technology
Intellectual Property
Therapeutic Research and Development



Biomedical research products
Primary human cells
Optimized cell culture media

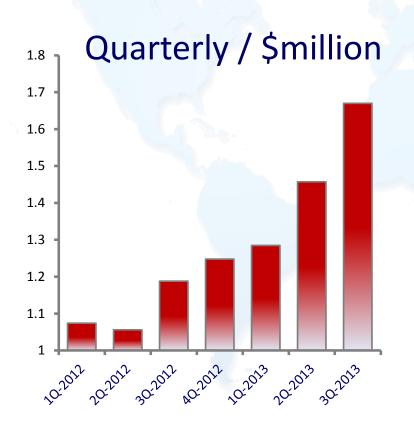


Branded anti-aging skin care products Proprietary stem cell skin care technology

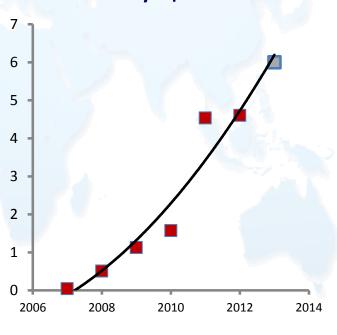


## **Commercial Operations**

# Strong Revenue growth from Biomedical businesses



### Annual / \$million





## Lifeline Cell Technology



### Vertically integrated human cell and media business

- \$2.3 billion world-wide market (bcc research 2009)
- Over 130 cells and optimized culture media products
- Branded and OEM products
  - Growing domestic sales force
  - International Distributors covering EU / Japan







### Lifeline Skin Care



## Branded anti-aging stem cell skin care products



Four Seasons Resort Maui at Wailea



Rosewood Tucker's Point Bermuda

Marriott
Palm Beach



Jen Galardi Hollywood Fitness guru and yoga instructor

Emme - Supermodel Woman of the year (Glamour Magazine) One of the 50 most beautiful people (People)



Listarted using Lifeline Skin Care Defensive Day Moisture Serum and Recovery Night Moisture Serum.

I see a difference with the fine lines slowly going away.

Enwe

Emme is the world's leading plus-siz model and committed to healthy skir body, mind and spirit.



Someday, stem cells will change the world.

Today, stem cells will change your skin.

Lifeline<sup>©</sup> speeds up production of new skin cells and stimulates your skin's ability to repair itself. Damaged skin cells causing fine lines and wrinkles and superficial imperfections are quickly outnumbered by the proliferation of healthy cells. You'll see an



What this company is doing is very exciting. And the products... they really work.

JOHN MAULDIN

READ MORE

John Mauldin

Economist and Best Selling Author



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Questions?

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