



**ALLIANCE** for  
*Regenerative Medicine*

*2013 Regenerative Medicine  
State of the Industry Briefing*

January 8, 2013

Geoff MacKay, Chairman, ARM

# *The Aging Global Population*



In the U.S., between 2010 and 2030 there will be a **~80% increase in the number of people age 65 or over.**

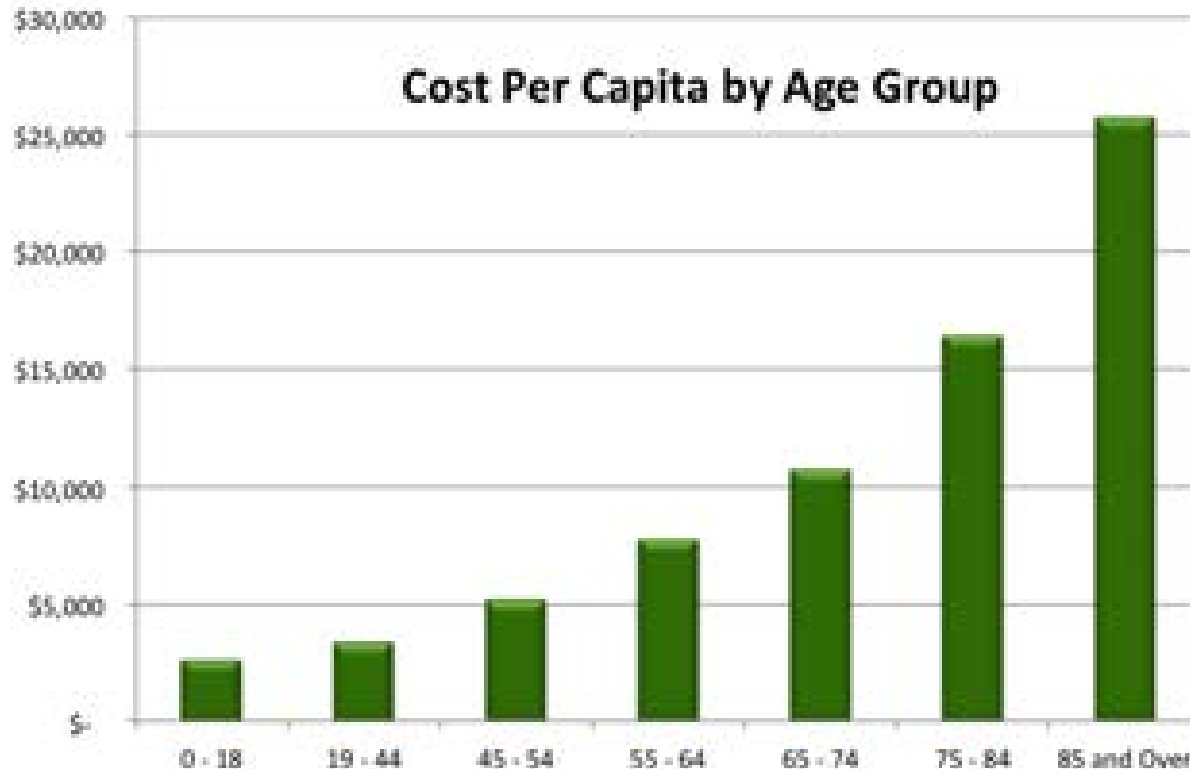
This segment of the population will increase from 40 million to more than 72 million individuals.

The challenge is just as great, or even greater in Europe and Asia.

# The Healthcare Economics Problem Created by an Aging Population

+ As we age, we spend more on healthcare due to the increased incidence of certain diseases.

**Average Annual U.S. Per Capita Healthcare Expenditures by Age**



## Key drivers in cost increases:

- ❖ Increased incidence, impact of chronic disease
  - Heart disease, cancer, stroke, pulmonary disease, diabetes, osteoporosis
- ❖ Significant burden associated with seriously ill
  - 1% of most seriously ill account for >25% total healthcare expenditures
- ❖ From 2006-2030, it is estimated that:
  - People in the age range 65-74 years will increase from 6% to 10% of the total population
  - People 75+ will increase from 6% to 9% of the total population and continue to grow to 12% by 2050

# The Aggregate Economic Impact of an Aging Population

+ If costs per capita remain constant over the next 20 years (i.e. NO INFLATION) the increase in aggregate healthcare costs will still be substantial.



Source: www.census.gov

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# *RegenMed and Key Therapeutic Areas*

+ *RM treatments have the potential to dramatically shift the cost curve for conditions that are more prevalent in an aging population.*

## **Major Areas of Need / Opportunity:**

- ❖ **Cardiovascular Disease** → Myocardial Infarction, Congestive Heart Failure, Vascular Disease / Critical Limb Ischemia
- ❖ **Hematopoietic Conditions** → Oncology/GVHD, Inherited Genetic Deficiencies
- ❖ **Inflammatory & Immune Disease** → Diabetes, IBD, RA, Lupus & others
- ❖ **Neurological Injury & Disease** → Stroke, TBI, Parkinson's, Multiple Sclerosis, Cerebral Palsy, Orphan Neurological Conditions, Spinal Cord Injury
- ❖ **Ocular Disease** → Age-related Macular, Stargardt's Macular Dystrophy, Retinitis Pigmentosum, Glaucoma, Corneal Transplants
- ❖ **Orthopedic Conditions** → Trauma, Age-related Degeneration
- ❖ **Pulmonary Disease** → COPD, ARDS
- ❖ **Renal Disease**

# *RegenMed and Key Therapeutic Areas: Statistics*

- + *The potential savings from regenerative medicine treatments for the United States in terms of reducing the direct costs associated with chronic diseases have been estimated at approximately \$250 billion a year.\**

## **Cardiovascular Disease**

- 100 million U.S. people afflicted
- \$316 billion – U.S. aggregate direct costs

## **Diabetes**

- 25 million U.S. people afflicted
- \$175 billion – U.S. aggregate direct costs

## **Stroke**

- 795 thousand U.S. people afflicted each year
- \$73 billion – U.S. aggregate direct costs

## **Alzheimer's**

- 35.6 million U.S. people living with the disease
- \$200 billion – U.S. aggregate direct costs

## **Age-related Macular Degeneration**

- 1.8 million U.S. people afflicted
- \$255 billion – global direct costs

## **Parkinson's Disease**

- 1 million + U.S. people afflicted
- \$23 billion – U.S. aggregate direct costs

## **Spinal Cord Injury**

- 275 million U.S. people afflicted
- \$40.5 billion – U.S. aggregate direct costs

## **Peripheral Arterial Disease (PAD)**

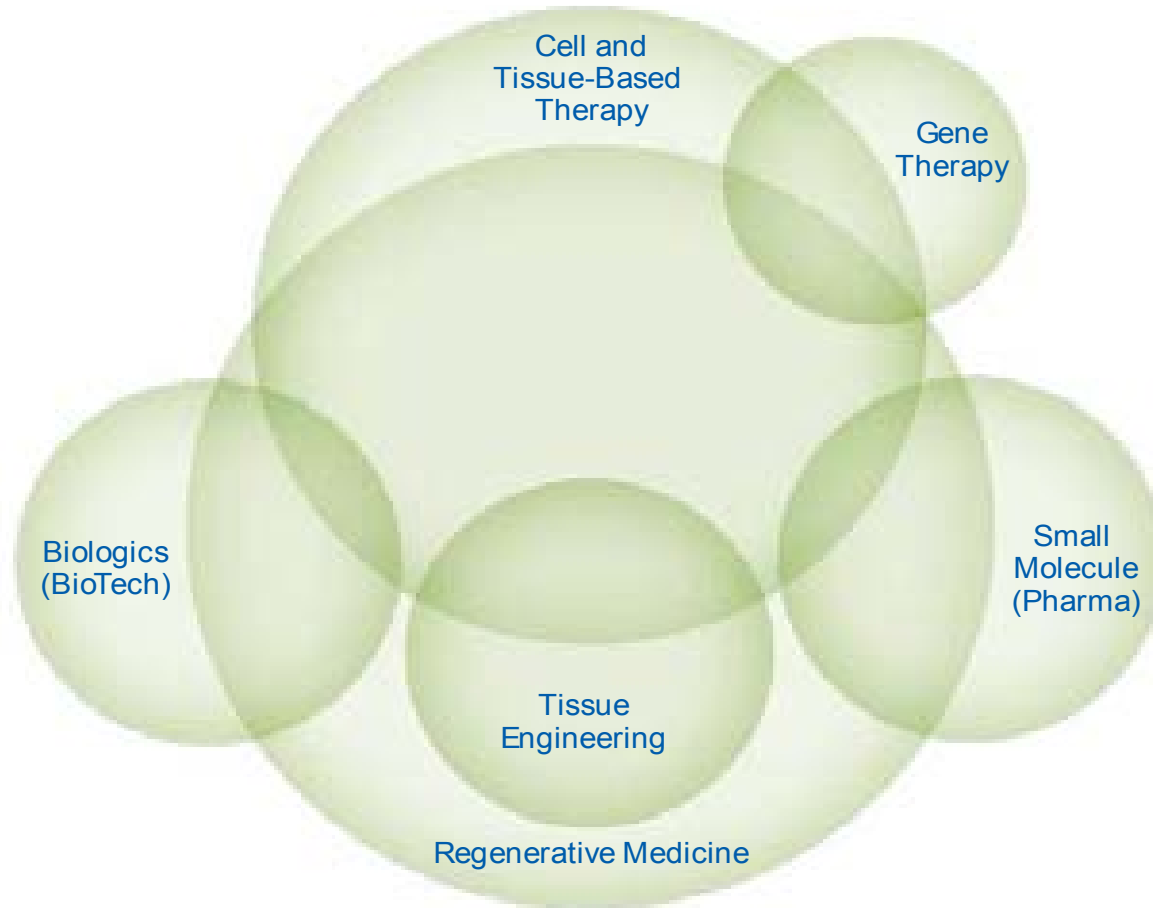
- 10 million U.S. people afflicted
- \$4.4 billion – U.S. aggregate direct costs

# *Technologies to Transform Healthcare*

- + **Regenerative Medicine (RM)** is a revolutionarily field focused on development and application of cell-based therapies & related technologies to enhance the natural healing process, and/or replace or regenerate organs and tissues. RM is focused on addressing the underlying clinical problem, and doing it cost effectively – not just providing palliative care.
- ❖ **Cell Therapy** — Using cells therapeutically to repair function to bodily tissue or organs. The two dominant forms of cell therapy are *autologous* (using one's own cells) and *allogeneic* (donated cells).
- ❖ **Tissue Engineering** — Creating new organs, and tissues to replace or repair existing organ function.
- ❖ **Medical Devices** — Using cell-based products for medical purposes in patients, in diagnosis, therapy or surgery.
- ❖ **Drug Discovery** — Using bioassays created from different cell sources to create highly accurate drug discovery platform models for understanding disease states, and for testing of traditional pharma compounds.
- ❖ **Small Molecules and Biologics** – Using RM technologies along with conventional drug discovery techniques to develop new small molecule and biologic drugs that can selectively modulate the body's own naturally occurring stem cells for therapeutic purposes.

# *Technologies to Transform Healthcare*

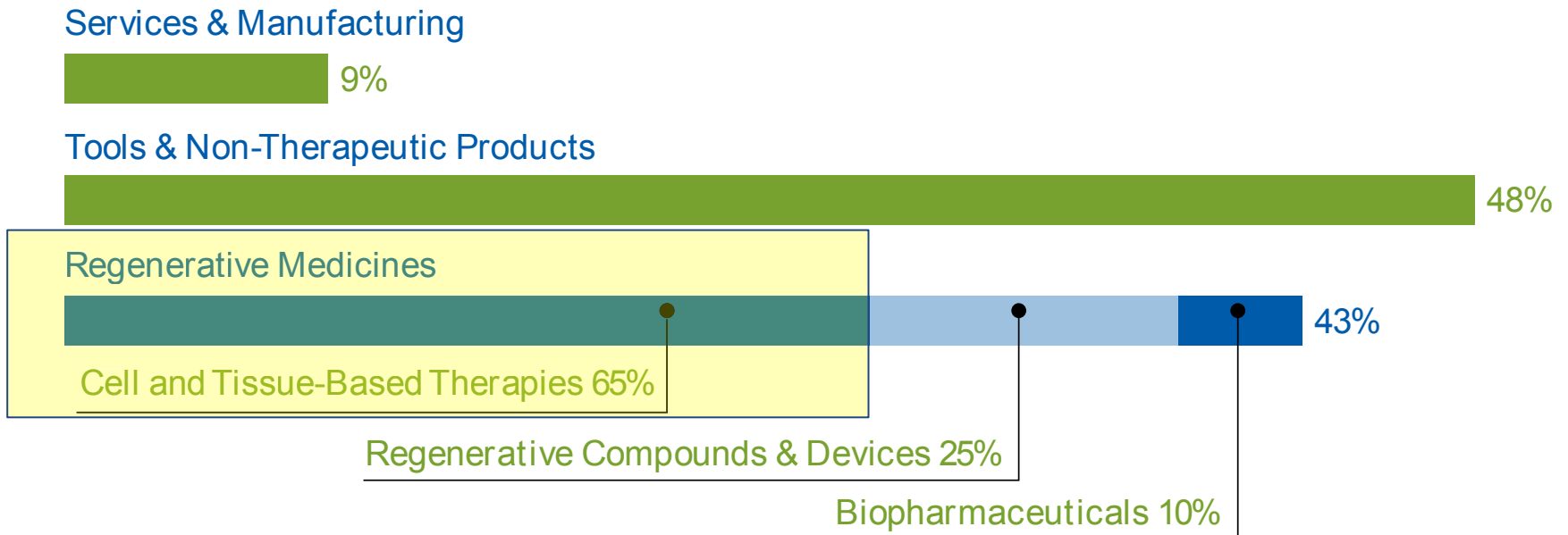
- + **Regenerative Medicine (RM)** is a revolutionary field focused on translating fundamental knowledge in biology, chemistry and physics into materials, devices, systems and a variety of therapeutic strategies which augment, repair, replace or regenerate organs and tissues.





# Regenerative Medicine Industry Overview

+ **The Regenerative Medicine (RM)** industry comprises service and manufacturing companies, tools and non-therapeutic products, cell and tissue based therapies, regenerative compounds and devices and biopharmaceuticals.



# *A Global Look at the Cell and Tissue Based Therapeutic Sector*

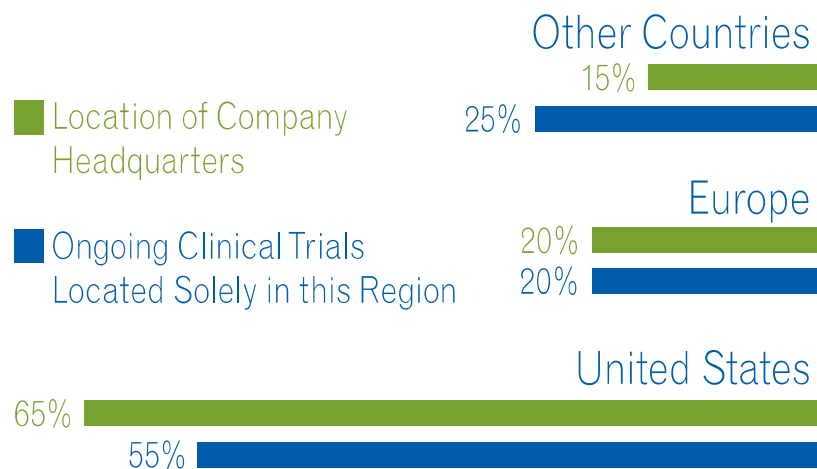
- Arguably the most prominent segment of the regenerative medicine industry, this sector includes more than 250 companies developing therapies for almost every imaginable disease or condition.
- There are approximately 300 cell and tissue-based therapeutics commercially available or in clinical development in countries with formal regulatory framework, 55 of which are described and marketed as regenerative medicine products.
- Research conducted by the Alliance valued the top 15 regenerative medicine products, based on revenue generation, to total the following amounts beginning in 2010:
  - \$460 million – 2010 (estimated)
  - \$730 million – 2011 (estimated)
  - \$900 million – 2012 (projected)
- All but one of these 15 products is for skin, wound, bone or cartilage repair, with the exception of Dendreon's Provenge, approved by the FDA in 2010 for late-stage prostate cancer. The first of these products was brought to market in 1998 and collectively these products have treated over 500,000 patients through the end of 2011.

# A Global Look at the Cell and Tissue Based Therapeutic Sector – Industry Snapshot

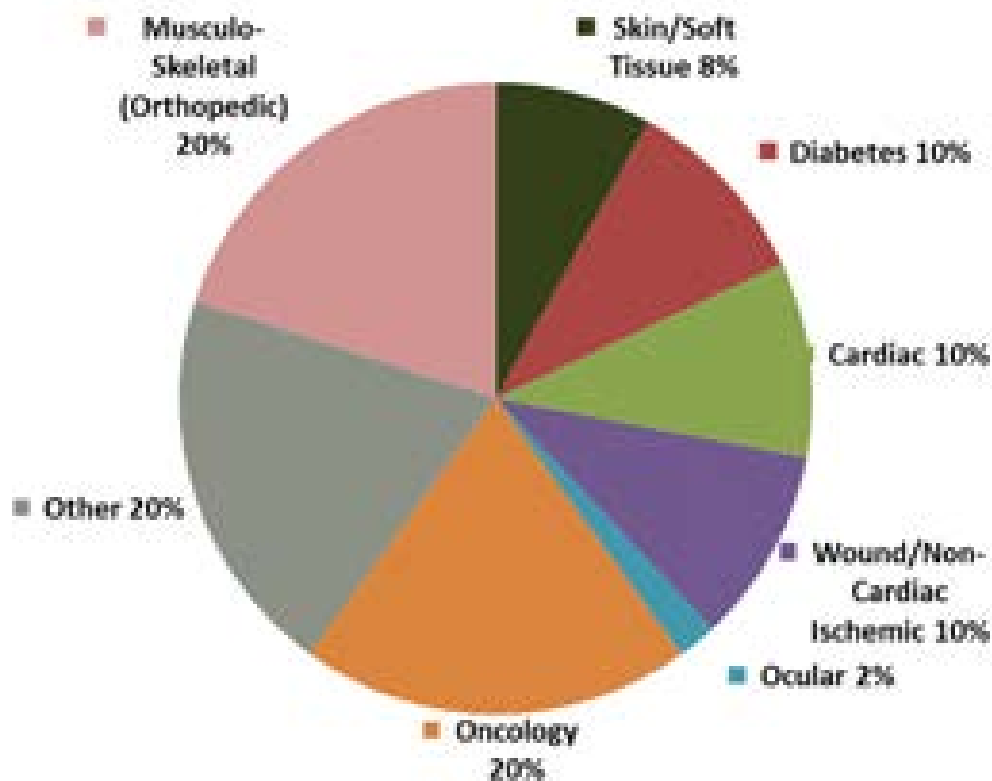
## Cell and Tissue-Based Segment

**25%:** Percentage of cell and tissue companies which are publicly traded.

## Companies and Trials by Location



## Therapies Being Sold or Tested



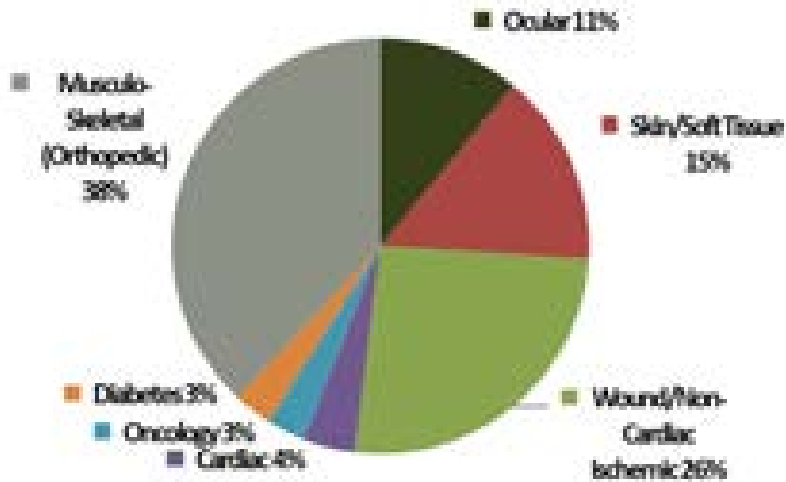
# *Multiple Products Already on the Market*

+ An estimated 500,000 patients have been treated with FDA approved cell therapy products.

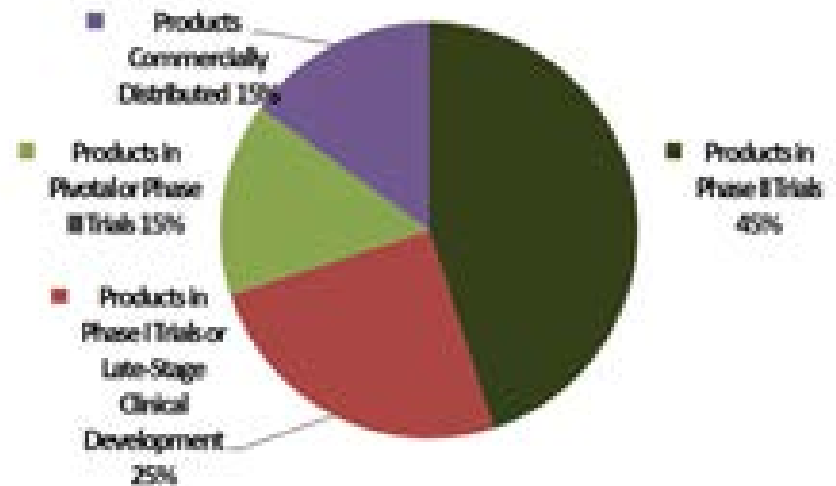


# A Global Look at the Cell and Tissue Based Therapeutic Sector – Clinical Trial Overview

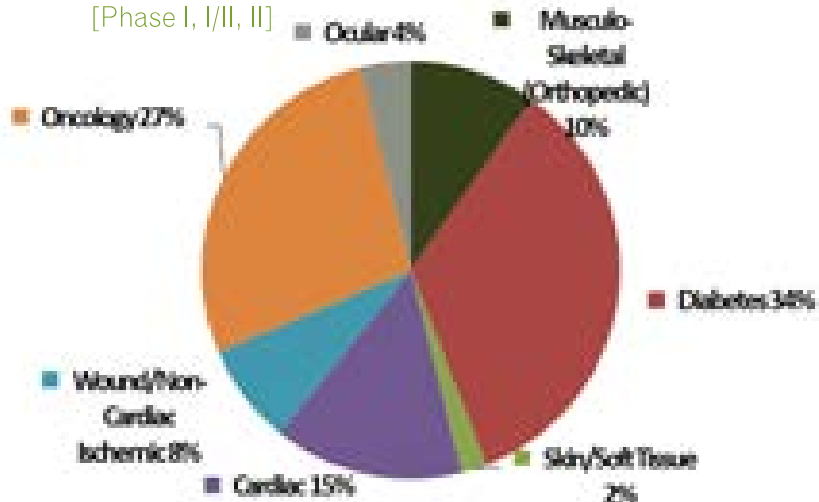
Products Commercially Available



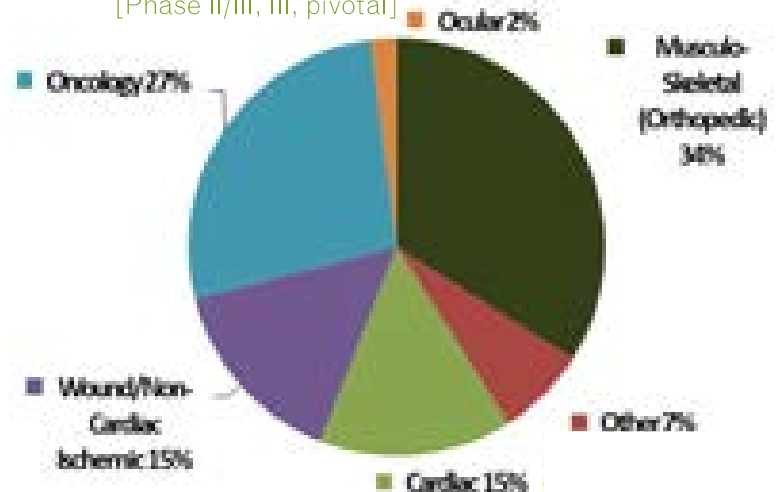
Clinical Trial Overview



Currently in Early-to-Mid Stage Trials  
[Phase I, I/II, II]



Currently in Late Stage Trials  
[Phase II/III, III, pivotal]



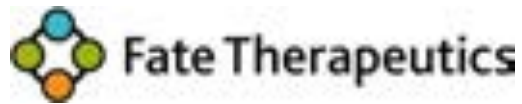
# *Major Companies with Regenerative Medicine Programs and/or Partnerships*

*Johnson & Johnson*



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*Regenerative Medicine*

# *Companies with Programs in Early- to Mid-Stage Clinical Development*



# *Companies with Programs in Mid- to Late-Stage Clinical Development*



AlloCure



avita<sup>medical</sup>



HEALTHPOINT<sup>BIOTHERAPEUTICS</sup>

ISTO<sup>Technologies, Inc.</sup>



TIGENIX<sup>Living Medicines</sup>

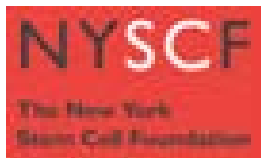
ALLIANCE<sup>Regenerative Medicine</sup>



# *Contract Manufacturers, Service Providers and Tools Companies Investing Heavily in RegenMed*



# Leading Research Institutions and Translational Centers Focused on Regenerative Medicine



# *Patient Advocacy Organizations and Foundations*



# Current Alliance Members

**+ There are currently over 125 member organizations, reflecting over 400% growth since 2009.**

## COMPANIES

Aastrom Biosciences, Inc.  
Aderans Research Institute  
Advanced Cell & Gene Therapy  
Advanced Cell Technology, Inc.  
Akron Biotechnology, LLC  
Aldagen/Cytomedix  
AlloCure  
AlloSource  
American CryoStem  
Amoryte  
Aposcience AG  
Athersys, Inc.  
AxoGen, Inc.  
Avita Medical  
Baxter  
Beckman Coulter, Inc.  
Becton Dickinson and Company  
Bell BioSystems  
BioLife Solutions, Inc.  
BioSpherix  
Capricor  
Celgene Corporation  
Cell Line Genetics, Inc.  
Cell Therapy Group  
Cellerant Therapeutics, Inc.  
CellGenix  
Cellular Dynamics International  
Cellular Technology Limited  
Celsense Inc.  
Circle Biologics  
Cytori Therapeutics Inc.  
DiscGenics  
EMD Millipore Corporation  
Equalix  
Fate Therapeutics  
Fibralign  
Fisher BioServices  
GE Healthcare  
Genzyme Corporation, a Sanofi Co.  
Harvard Bioscience, Inc.  
Healthpoint Biotherapeutics  
HemoGenix  
Histogenics  
Humacyte, Inc.  
Intercytex  
Invetech Pty. Ltd.  
InVivo Therapeutics  
iPierian Inc.  
ISTO Technologies  
Johnson & Johnson  
Juventas Therapeutics  
Life Technologies Corporation  
Lonza Group Ltd.  
MaxCyte  
Medistem Inc.  
Medpace, Inc.  
Mesoblast  
MiMedx  
Nanofiber Solutions  
NeoStem, Inc.  
Organogenesis Inc.  
Organovo, Inc.  
Owl Biomedical  
Pathfinder Cell Therapy, Inc.  
PCT  
Pfizer Inc.  
Pharmacell  
Pluristem Therapeutics

Q Therapeutics  
Regen BioPharma  
ReNeuron Group plc  
RhinoCyte, Inc.  
Roche  
RxGen  
Sangamo BioSciences, Inc.  
Shire Regenerative Medicine  
SironRX  
Sistemic Scotland Limited  
Stem Cell Media, LLC  
StemBioSys, LLC  
StemCells, Inc.  
TAP Biosystems  
Tarix Pharmaceuticals LTD  
Tengion  
Terumo BCT, Inc.  
TiGenix  
Tissue Genesis, Inc.  
UroTiss  
ViaCyte

## RESEARCH INSTITUTIONS

California Institute For Regenerative Medicine  
Cleveland Clinic  
Centre for Commercialization and Regen Med  
Johns Hopkins Translational Tissue Engineering  
Northwestern, Comprehensive Transplant Center  
Neural Stem Cell Institute  
Pittsburgh Tissue Engineering Initiative  
Sanford Burnham Medical Research Institute  
Texas Heart Institute  
UC-London, Centre for Stem Cells & RM  
U.Louisville, Cardiovascular Innovation Institute  
Wake Forest Institute for Regenerative Medicine

## FOUNDATIONS/ASSOCIATIONS

ACRO  
Alpha-1 Foundation  
ALS Association  
Californians for Cures  
Cell Society  
Friends of Cancer Research  
Genetics Policy Institute  
Human Organ Project  
JDRF  
Missouri Cures  
Nebraska Coalition for Lifesaving Cures  
New York Stem Cell Foundation  
Parkinson's Action Network  
Regenerative Medicine Foundation  
South Texas Blood and Tissue Center  
Student Society for Stem Cell Research  
Texas Cures Education Foundation  
Unite 2 Fight Paralysis

## INVESTORS

Asset Management Company  
Kentucky Seed Capital Fund  
Novitas Capital  
Tekesta Capital Partners  
Triathlon Medical Ventures

# *The Big Picture – View From the Front Lines*

## **2013 Regenerative Medicine State of the Industry Briefing Panelists**

- ❖ **Susan Solomon**, CEO, New York Stem Cell Foundation
- ❖ **Jeff Jonas**, President, Shire Regenerative Medicine
- ❖ **Matthias Steger**, Global Head Research & Technology Partnering, Roche Ltd.
- ❖ **Robert Palay**, CEO, Cellular Dynamics International
- ❖ **Robert Shaw**, Commercial Director - Stem Cell Initiative, EMD Millipore
- ❖ **Chris Calhoun**, CEO, Cytori Therapeutics
- ❖ **Karin Hehenberger**, EVP and CMO, Coronado Biosciences
- ❖ **Mark Frohlich**, EVP Research & Development and CMO, Dendreon
- ❖ **Gil Van Bokkelen**, Chairman & CEO, Athersys, Inc.



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