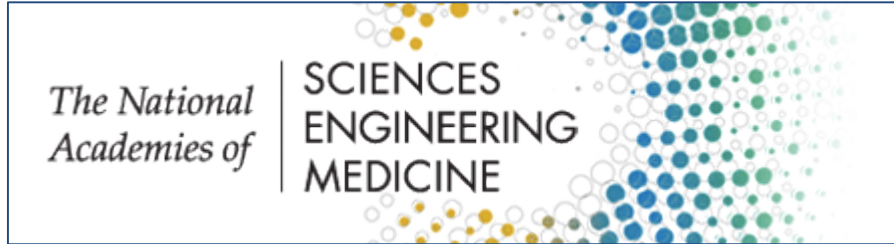
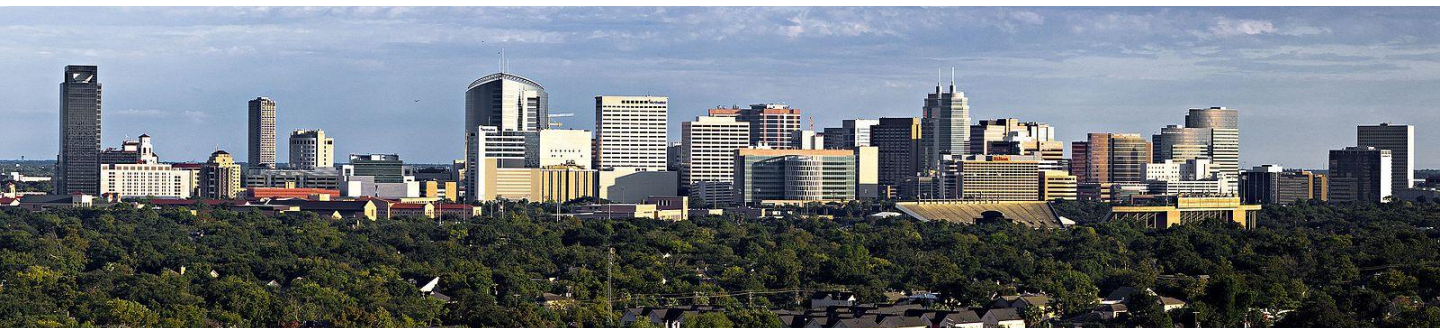


Navigating the Manufacturing Process & Assuring the Quality of Regenerative Medicine Therapies

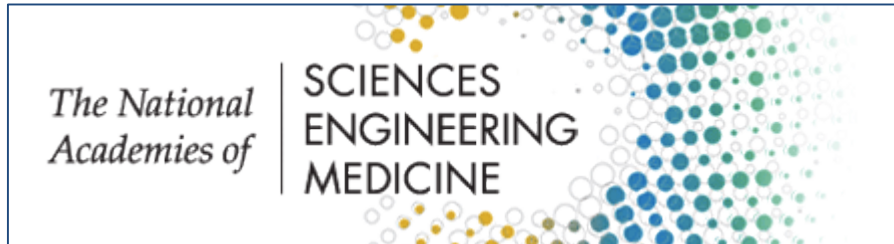
A Workshop



Adrian Gee
Center for Cell & Gene Therapy
Baylor College of Medicine
Houston, Texas

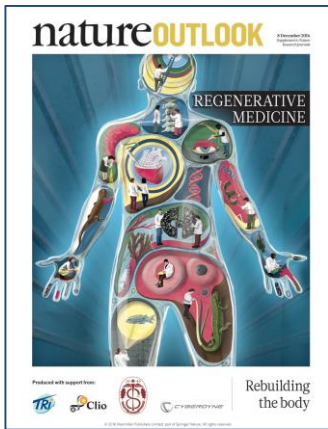


Thank you for the Invitation



I have no disclosures to make

The Promise



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Type 1 Diabetes Cure On The Horizon?
Scientists Use Stem Cells To Produce Billions
Of Insulin-Producing Beta Cells



Cure for Type 1 diabetes imminent after Harvard stem-cell breakthrough

Harvard University has produced the vast quantities of insulin-producing cells

Stem Cell Breakthrough Puts Type 1 Diabetes Cure In Reach

By Carl Engeling | October 10, 2014 1:35 pm

Stem-cell cure for Type 1 diabetes 'on par with discovery of antibiotics'



PBS NEWSHOUR

TOPICS • HEALTH

Experimental therapy trains immune cells to hunt and kill blood cancers

March 17, 2012 4:02 PM EDT

Dr. Carl June
ONCOLOGIST, PERELMAN SCHOOL OF MEDICINE

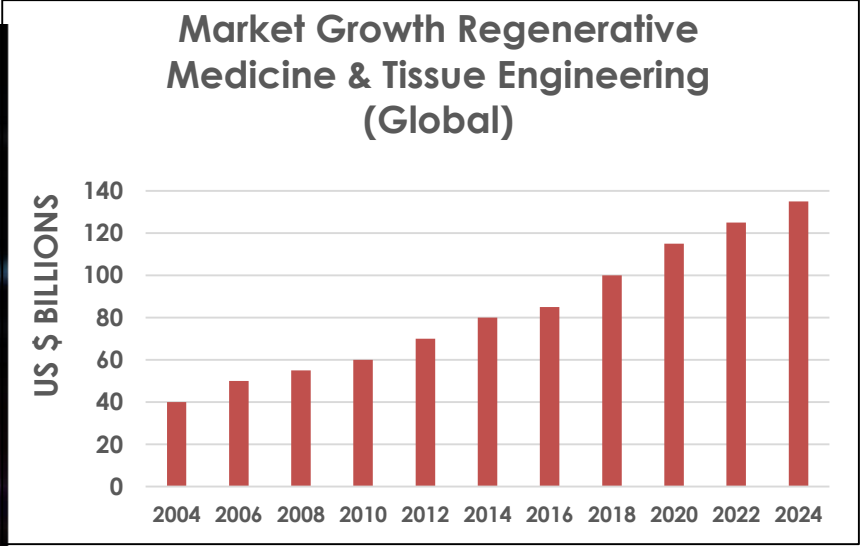
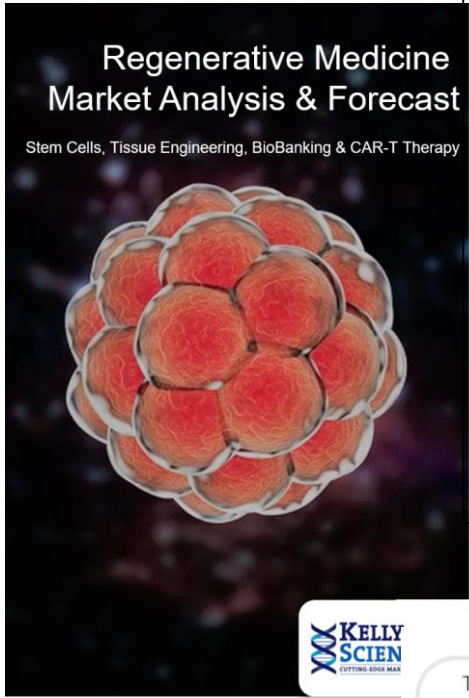
At the University of Pennsylvania, a research team has been working on an experimental treatment to kill leukemia with a patient's own immune system cells. So far, the results have shown startling success. Special correspondent Jackie Judd reports on the growing research on immunotherapy in fighting cancer.

RELATED

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The Market - \$140BN by 2024



7th International Congress on Tissue Engineering & Regenerative Medicine

UK Growth Opportunities

The regenerative medicine market is expected to create **15,000 jobs** by 2020

2020 15,000 jobs

The synthetic biology global market is expected to reach **£62bn** by 2020

2020 £62bn

The Hype



Unproven Stem Cell Clinics Proliferate in the U.S.

tes advertise therapies for sports injuries, autism and MS via direct-to-consumer marketing

By Dina Fine Maron on June 30, 2016

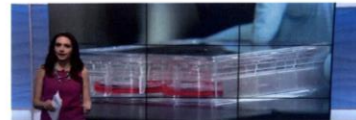


Desperate patients and false hope: a troubling trend for stem cell-based therapies

JUNE 4, 2015 TOED GUERNICOFF

A gambler's odds are usually stacked against them but the possibility, however slim, of hitting the jackpot keeps bringing them back to the table. Now imagine, unbeknownst to them, the system is rigged so there's a zero percent chance of any winnings. They'd essentially be giving their money away based on a false hope. Sadly, many desperate people looking for stem cell cures do exactly that.

Earlier this week, Cristin Severance, a Team10 TV news reporter in San Diego, investigated local stem cell clinics promising treatments for a number of chronic incurable diseases. Severance cites Stemgenex of La Jolla, which offers people with Parkinson's disease the chance of improving their symptoms through a therapy using stem cells from their own fat. This opportunity comes at a cost - \$15,000. According to stem cell expert Jeanne Loring of The Scripps Research Institute, there's no prospect the treatment will work.



The Regulatory Challenge

Risks-versus-Opportunity

Welcome or Not, FDA Focuses on Stem Cell Treatments

AUGUST 14, 2018 BY AGRON BY COLLEEN, WHAT'S IMPORTANT NOW -- AN FBI FOLLOWUP BY SAN FRANCISCO



HEALTH FDA moves to crack down on unproven stem cell therapies

BY DENA LEE MCFARLING @denaleemcfarling / FEBRUARY 6, 2018



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Cellular & Gene Therapy Products

Approved Products

Cloning

Regenerative Medicine Advanced Therapy Designation

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As described in Section 3033 of the 21st Century Cures Act, a drug is eligible for regenerative medicine advanced therapy (RMAT) designation if:

- a. The drug is a regenerative medicine therapy, which is defined as a cell therapy, therapeutic tissue engineering product, human cell and tissue product, or any combination product using such therapies or products, except for those regulated solely under Section 361 of the Public Health Service Act and part 1271 of Title 21, Code of Federal Regulations;

U.S. Department of Health and Human Services

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Upcoming Workshops, Meetings & Conferences (Biologics)

Final Agenda: Part 15 Hearing: Draft Guidances Relating to the Regulation of Human Cells, Tissues, or Cellular or Tissue-Based Products

My Task

To raise some of the issues faced by stakeholders trying to develop & license cellular & regenerative therapies



The Stakeholder Challenge

Ideal Product

- Starting material should be easy to collect (or generate)
- Manufacturing using automated simple closed systems
- Rapid, predictive testing methods
- Off-the-shelf product
- Long shelf life under simple conditions
- Product easy to distribute & administer



The Stakeholder Challenge

Aim: High Quality & Low Cost

- Collection
- Manufacturing
- Testing & Release
- Storage
- Transportation



The Stakeholder Challenge

Collection

- Increasing range of starting cells
- Appropriate donor testing?
- Varying risks of collection methods
- Ancillary agents used for collection
- Variability in material obtained due to donor & collector
- Transport to manufacturing site



The Stakeholder Challenge

Manufacturing

- Allogeneic-versus-Autologous products
- Approved media & ancillary reagents & devices e.g. scaffolds
- Closed systems at all stages of manufacturing
- Availability of approved manufacturing hardware with inbuilt monitoring



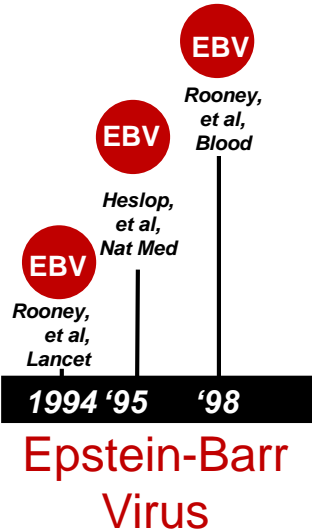
The Stakeholder Challenge

Manufacturing

- Easy scale-up & scale-out
- Integrated software for GMP operations e.g. QA, QC, document management etc.
- Scarcity of staff
- Staff training & certification programs?



Virus-specific T cells



Used across HLA
Barriers without GvHD

Off
the
Shelf

The Stakeholder Challenge

Testing & Release

- New rapid testing assays (e.g. sterility)
- Lack of potency assays that correlate with clinical efficacy
- Cost of testing e.g. for viral vectors
- Need development & regulatory approval of new release tests
- Standardization of assays with common controls



The Stakeholder Challenge

Storage

- Effects of cryostorage on stability & potency
- Development of new “holding” techniques
- Methods to avoid product manipulation upon receipt at clinical sites



The Stakeholder Challenge

Distribution/Transportation

- Standardized labeling – ISBT 128?
- Improvements on dry shipper method
- “Just-in-time” fresh cell shipments
- Improvements in formulation & packaging to facilitate shipment and administration



The Stakeholder Challenge

Regulations & Standards

- Evolving regulatory environment – appropriate for these products?
- Interface between regulations & professional standards?
- Need for training programs?
- Balancing safety & efficacy versus patient access and demand



The Stakeholder Challenge

Other Issues

- Costs-versus-Charges for licensed products?
- Ability to pay?
- Funding of next generation efforts & non commercially attractive diseases?
- Longer term role for “academic” manufacturers?



Thank You!



Let the Discussions Begin!

*The National
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