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

The National Academies of SCIENCES ENGINEERING MEDICINE

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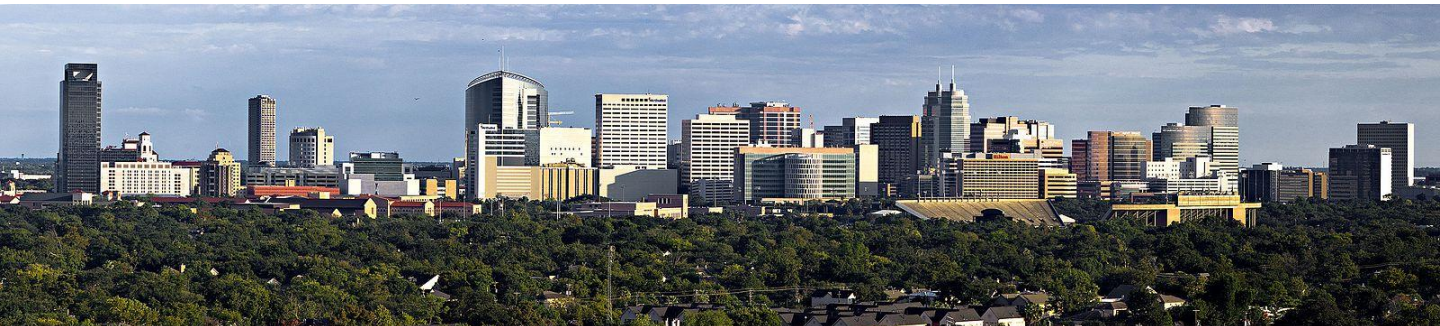
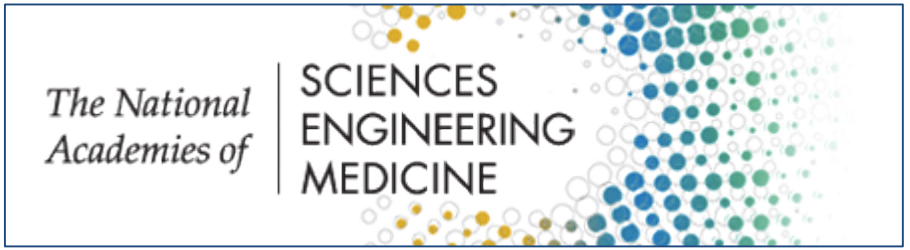
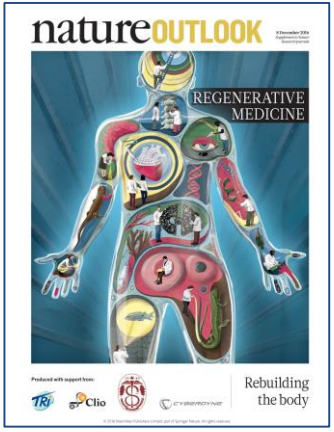


Thank you for the Invitation



I have no disclosures to make

The Promise


nature OUTLOOK
REGENERATIVE MEDICINE
Rebuilding the body


Science
SPECIAL ISSUE
REPAIR AND REGENERATION


The Telegraph
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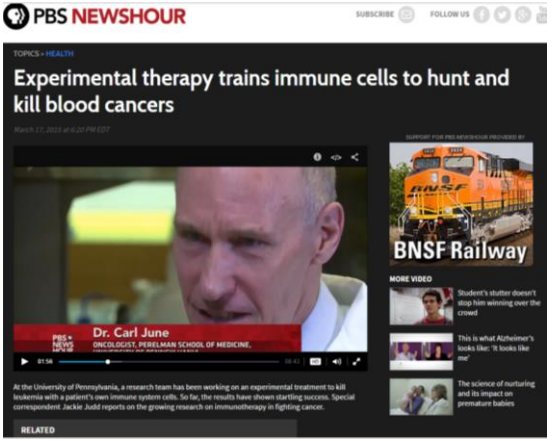
Cure for Type 1 diabetes imminent after Harvard stem-cell breakthrough
Stem Cell Breakthrough Puts Type 1 Diabetes Cure In Reach
By Carl Engelking | October 10, 2014 1:35 pm
Stem-cell cure for Type 1 diabetes 'on par with discovery of antibiotics'
STOP
DIABETES


TIME
SPECIAL HEALTH DOUBLE ISSUE
THIS BABY COULD LIVE TO BE 142 YEARS OLD
Dispatches From the Frontiers of Longevity


PBS NEWSHOUR
Experimental therapy trains immune cells to hunt and kill blood cancers
Dr. Carl June
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MORE VIDEO
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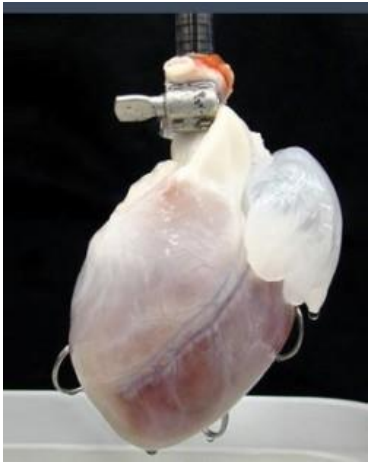
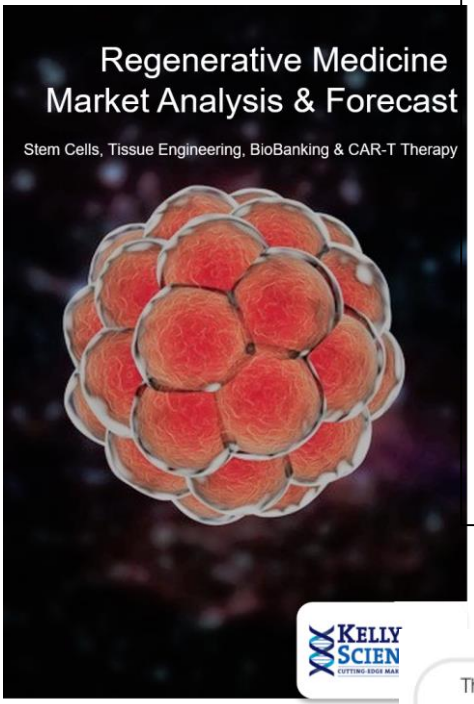
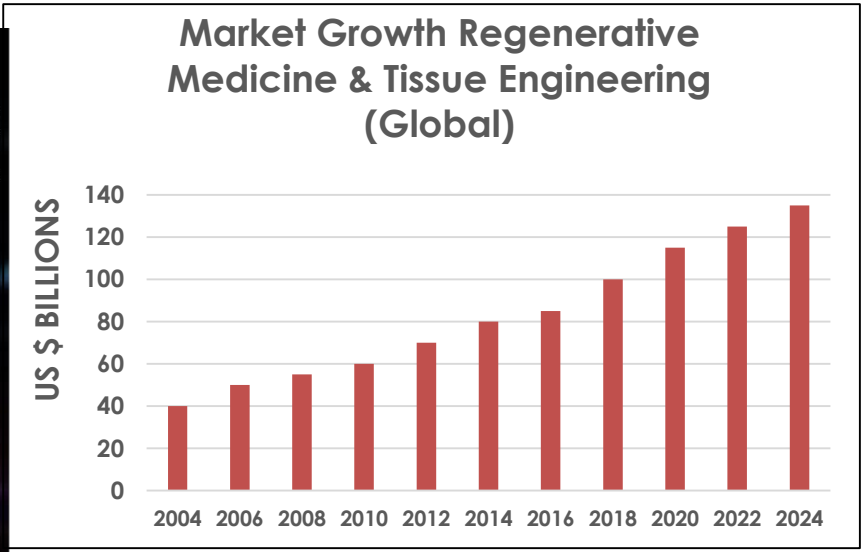


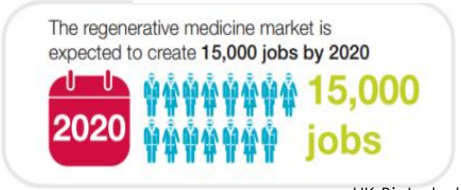
The Market - $140BN by 2024

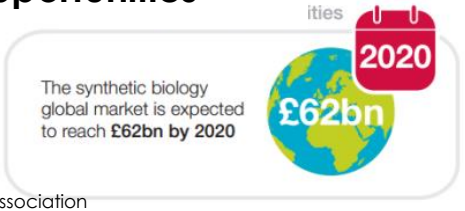




7th International Congress on Tissue Engineering & Regenerative Medicine

UK Growth Opportunities





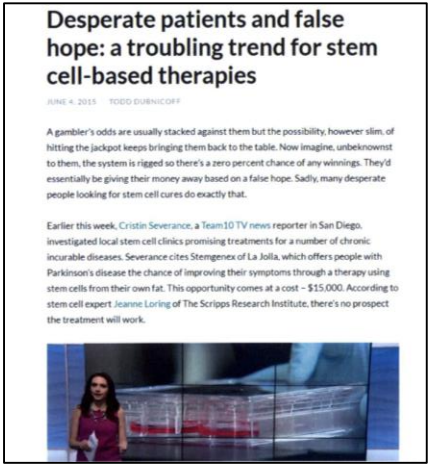
UK BioIndustry Association

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



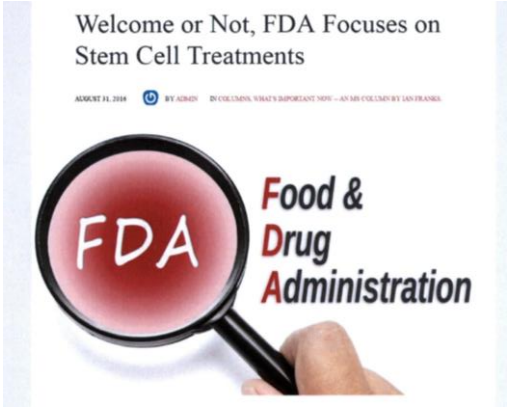
Desperate patients and false hope: a troubling trend for stem cell-based therapies

JUNE 4, 2015 TODD DUBNICOFF

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



My Task

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

The Stakeholder Challenge

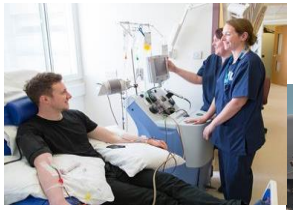
Ideal Product

- 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

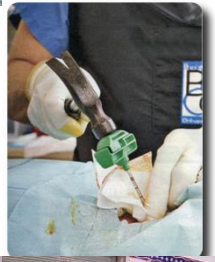
Aim: High Quality & Low Cost

- 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

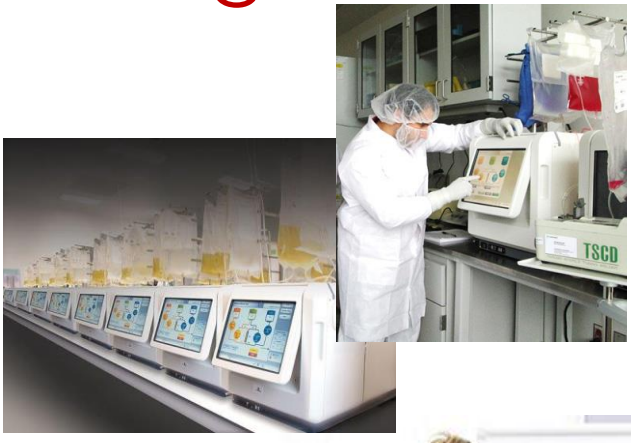


The Stakeholder Challenge
Manufacturing

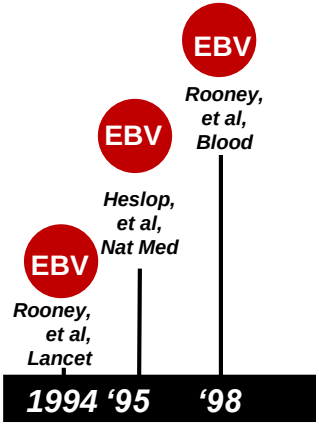
- Allogeneic-versus-Autologous products
- Approved media & ancillary reagents & devices e.g. scaffolds
- Closed systems at all stages of manufacturing
- Availability of approved manufacturing hardware with inbuilt monitoring

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



Epstein-Barr Virus

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?

AABM
ASSOCIATION OF ACADEMIC BIOLOGICS MANUFACTURERS

Thank You!

Let the Discussions Begin!

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