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

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Thank you for the Invitation

I have no disclosures to make
The Promise
The Market - $140BN by 2024

Regenerative Medicine Market Analysis & Forecast
Stem Cells, Tissue Engineering, BioBanking & CAR-T Therapy

Market Growth Regenerative Medicine & Tissue Engineering (Global)

US $ BILLIONS


7th International Congress on Tissue Engineering & Regenerative Medicine

UK Growth Opportunities

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

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

Unproven Stem Cell Clinics Proliferate in the U.S.

Desperate patients and false hope: a troubling trend for stem cell-based therapies
Welcome or Not, FDA Focuses on Stem Cell Treatments

FDA moves to crack down on unproven stem cell therapies

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,

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

Epstein-Barr Virus

Used across HLA Barriers without GvHD
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 Academies of
SCiences Engineering Medicine