Realizing the Promise of Regenerative Medicine and Upholding Public Trust: The Need for Rigorous Science and Data Sharing

NIH PERSPECTIVE

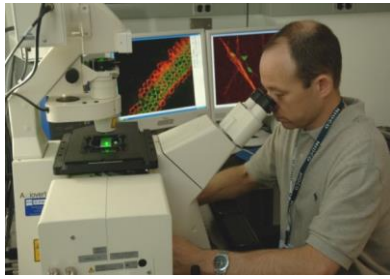
Congressional Briefing on
Regenerative Medicine Cell Therapies

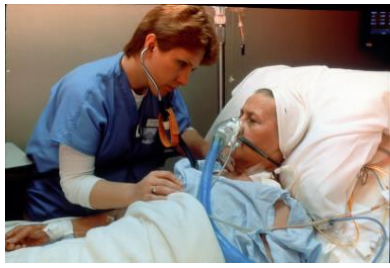
September 19, 2019
12:00 PM – 1:30 PM
902 Hart Senate Office Building

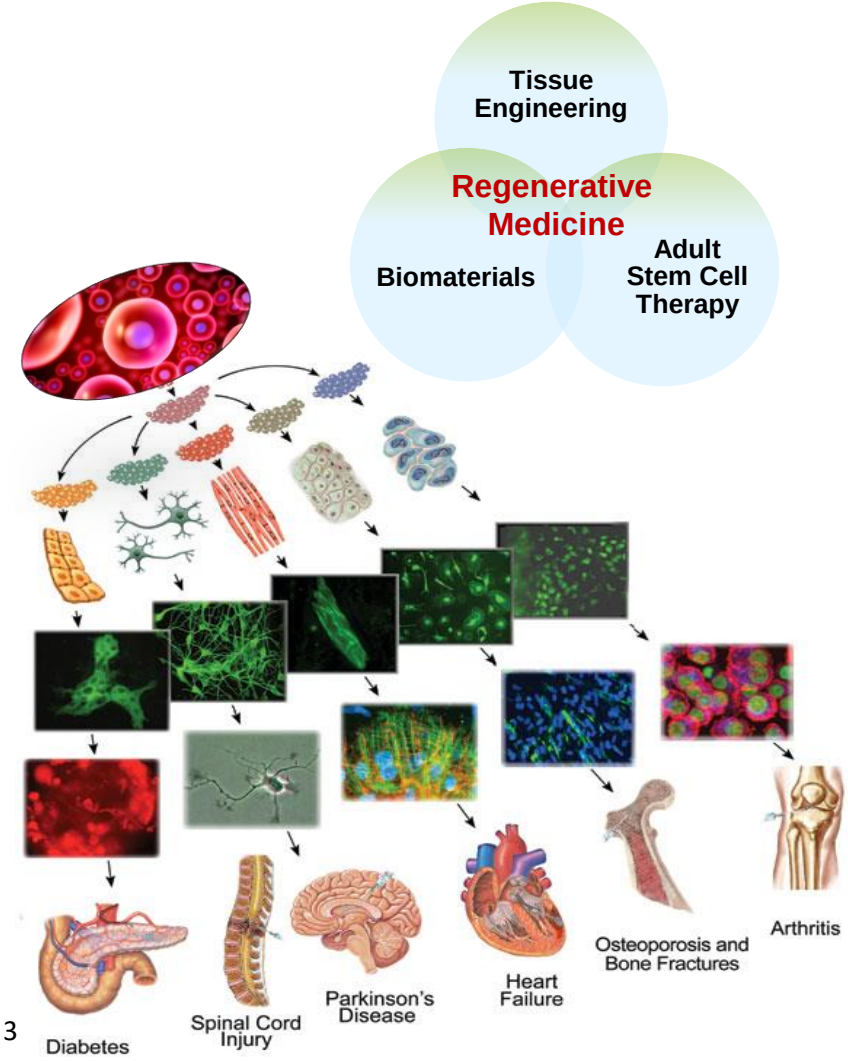
National Institutes of Health

NIH: Mission, Roles, Principles

- **Mission:**
  - To seek fundamental knowledge about the nature and behavior of living systems and to apply that knowledge to enhance health, lengthen life, and reduce illness and disability
- **Roles:**
  - Fund and conduct research
  - Stimulate and lead avenues of scientific inquiry
  - Serve as responsible stewards through peer review and oversight
- **Principles:**
  - Scientific rigor and reproducibility
  - Scientific integrity in conduct and reporting of research
  - Sharing of research data, materials, and other resources
  - Transparency and accountability
  - Engagement of the research community, patients, and other public sectors





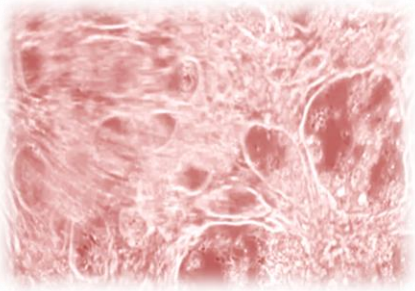


Regenerative Medicine

- Aims to repair or replace cells and tissues damaged by injury, disease, or aging
- Includes a wide range of technologies such as engineered biomaterials and tissues as well as gene editing or replacement/addition
- **The Promise:**
  - Delivery of therapeutic cells that restore normal structure and function
  - Leverage and enhance the body's own innate healing capacity

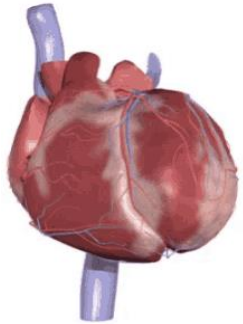
Promise of Regenerative Medicine: Example

Still Many Challenges



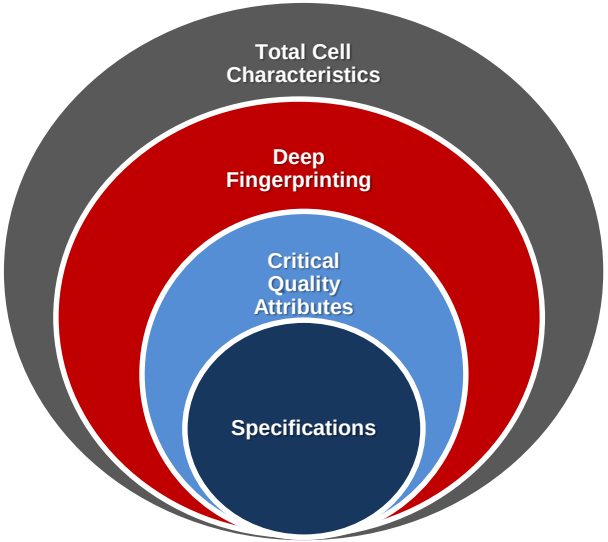
Cardiomyocytes derived from Human iPSC contract *in vitro* (Cell Applications Inc.)

Proof Pending:
Safety and Efficacy

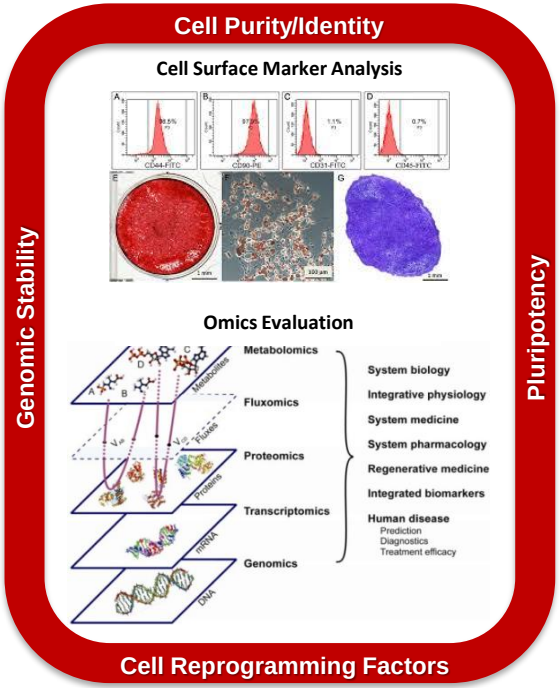


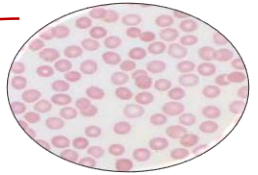
In-depth Cell Characterization → **Preclinical Studies** → **Clinical Trials** → **Data Sharing**

In-depth Cell Characterization



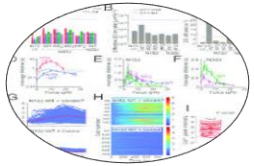
Levels of Cell Product Characterization



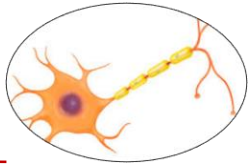


Morphology

Biochemical Attributes



Biophysical Attributes

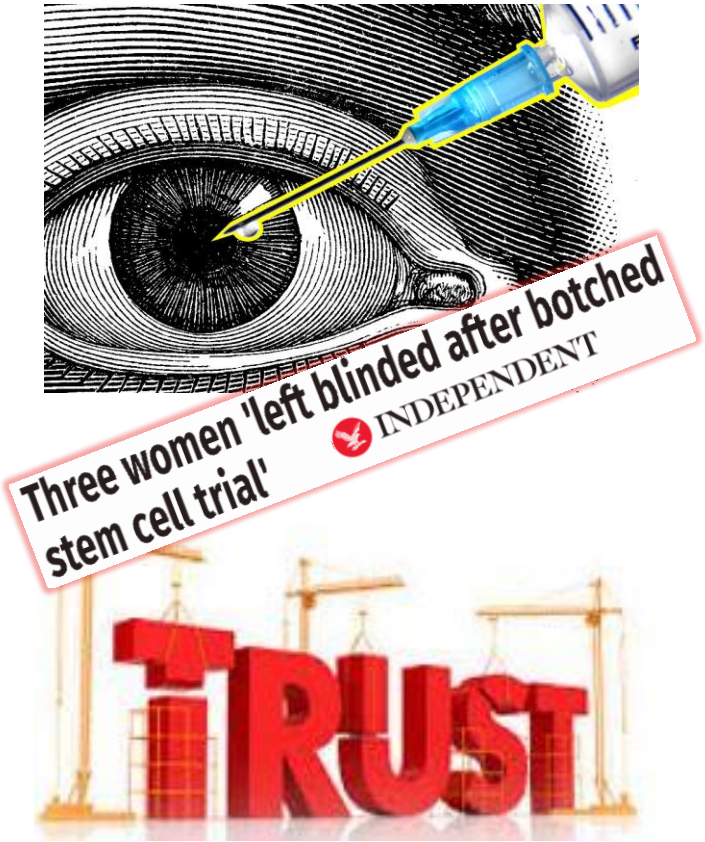


Function

National and International Expert Consensus

- **State of the science and clinical applications of RM:**
  - **While cell therapies offer potential promise, much must be learned about cell function and product manufacturing before safe and effective products can be administered in the clinic**

Upholding Public Trust



Importance of rigorous science and avoidance of "hype"

- Considerable hype in marketing; untested and unapproved RM products advertised
  - A number of trials are not under IND
  - "Pay-to-participate" clinical trials
- Harms to individuals from using such products have occurred; may lead to loss of public trust in:
  - The field of RM
  - Biomedical research in general

21st Century Cures Act: Regenerative Medicine

21st Century Cures

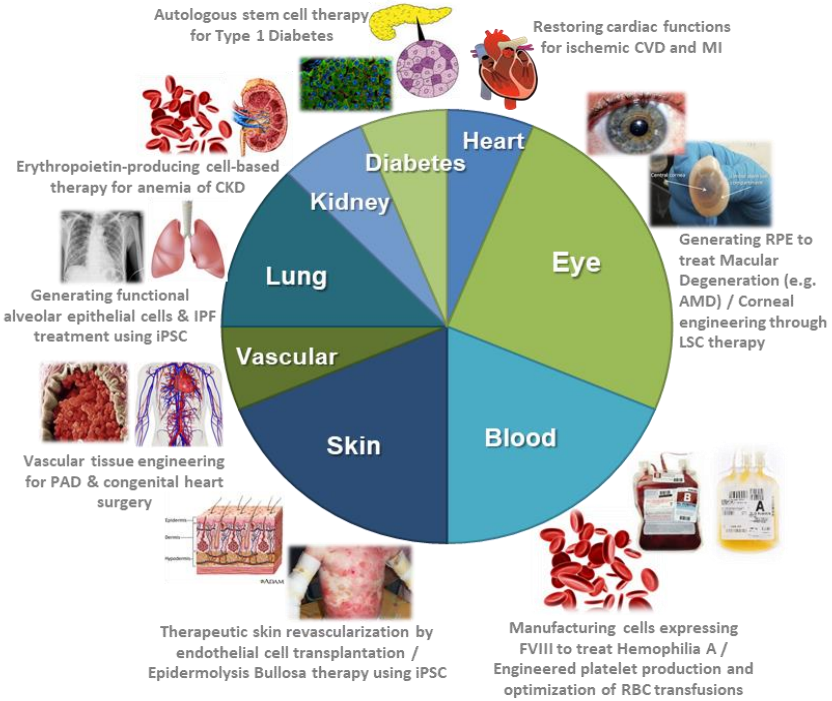
- **RM provisions applicable to NIH, FDA, and NIST**
- **Themes:**
  - Accelerating progress
  - Scientific rigor
  - Appropriate regulatory oversight and standards
  - Stimulating innovation and partnership
- **Established RM Innovation Project (RMIP)**
  - NIH, working in coordination with FDA, to support research to advance the field of regenerative medicine using adult stem cells
  - Funding authorized 2017-2020

Regenerative Medicine Innovation Project: Strategy

To significantly accelerate the field of RM through:

- **Funding late-stage pre-clinical and IND/IDE-enabling studies as well as carefully selected clinical trials that exemplify:**
  - **Rigorous science**
  - **Optimal regulatory compliance**
  - **Enhanced data sharing**
- **Providing resources necessary for investigators to address critical challenges identified through broad consultation**
- **Developing an innovative model for the support and conduct of RM research**

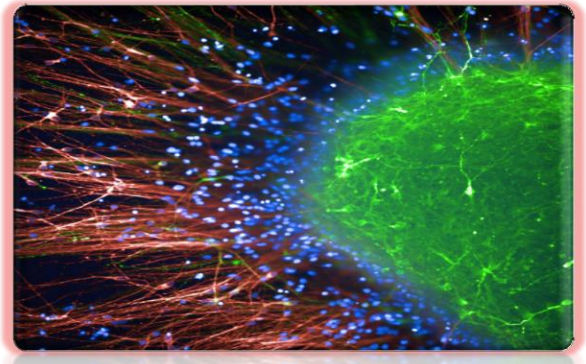
Current RMIP Portfolio: Clinical Indications



Understanding Scientific Challenges in RM

NIH-FDA co-hosted a multi-sector workshop to identify and discuss solutions to major scientific barriers in RM

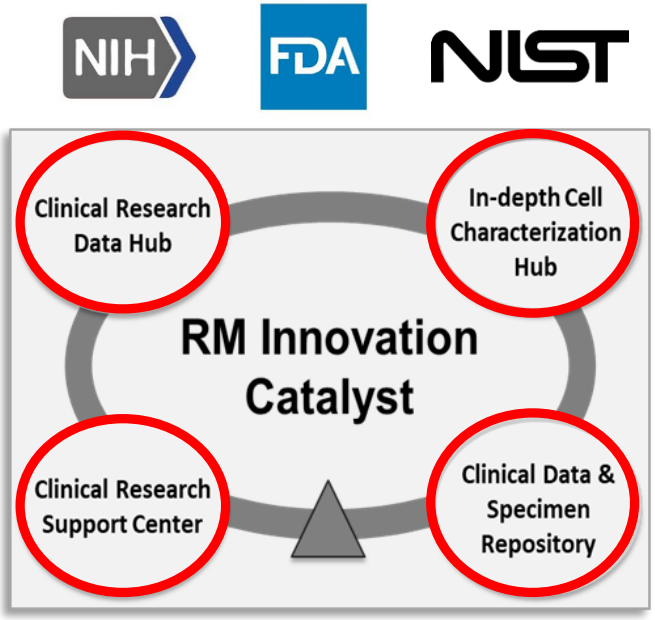
- **Several universal challenges emerged:**
  - **Need for regulatory support to enable development of robust IND/IDE applications**
  - **Transitioning from laboratory- to clinical-grade manufacturing of cell products**
  - **Limited understanding of the identity and nature of RM cell products used in the clinic**
  - **Limited availability of standardized data for analysis across studies hampers advancement of the field**



Accelerating Innovation: Addressing Challenges

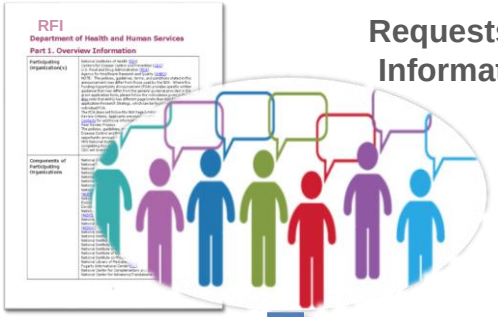
Regenerative Medicine Innovation Catalyst (RMIC)

- **NIH-FDA-NIST collaboration, in consultation with RM community**
- **Network of academic and private sector entities being launched to:**
  - **Provide manufacturing support and regulatory "coaching"**
  - **Apply advanced technologies for in-depth cell characterization**
  - **Develop data standards and common data elements**
  - **Promote collection and sharing of standardized cell product and clinical outcomes data**



➢ ***A pilot for a new model for support and conduct of RM clinical research***

Consultative Approach to Advancing RM Clinical Research



Requests for Information

Further Expert Multi-Sector Scientific Input

FDA U.S. FOOD & DRUG ADMINISTRATION

NIH National Institutes of Health

NIST National Institute of Standards and Technology U.S. Department of Commerce

Strategic and Tactical Framework for Advancing RM Clinical Research

Advisory Groups

Workshops

Accelerating the Development of Safe and Effective Cell-based Treatments

- Promote and support rigorous science and regulatory compliance
- Assist investigators in developing clinical-grade cell products
- Standardize and collect in-depth cell characterization data and individual level participant data
- Use common vocabularies and provide a cloud-based platform for sharing, integration, and analysis of product and clinical data

- Advances the field, promotes public trust
- Optimizes ability to manufacture cells that will produce the intended clinical effects
- Enables deeper understanding of the composition and function of stem cell products and correlation of cell characteristics with clinical outcomes
- Enables analyses across studies

Realizing the promise of Regenerative Medicine and upholding public trust

- Rigorous science
- Attention to safety
- Accountability
  - Stewardship and oversight
- Transparency
  - Broad sharing of both cell product and clinical outcomes data