

# The 21<sup>st</sup> Century Cures Act: Implementation of Regenerative Medicine Provisions

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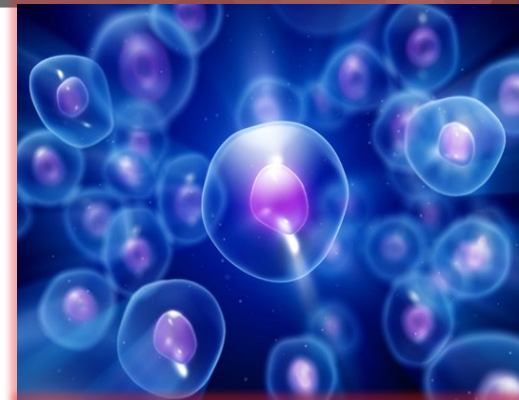
**NIH Advisory Committee to the Director**

**March 28, 2017**



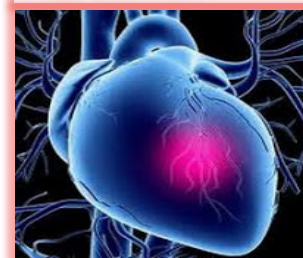
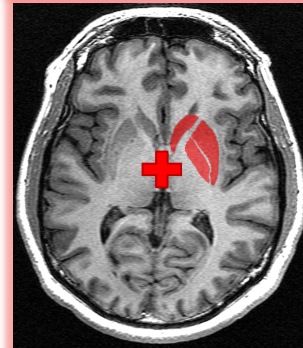
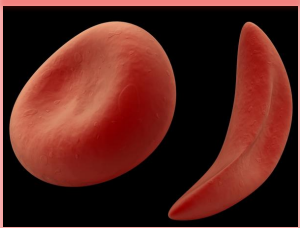
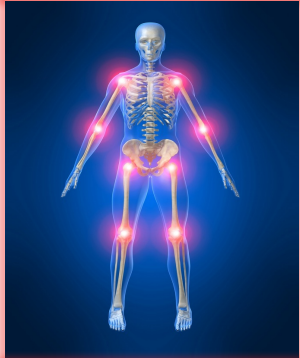
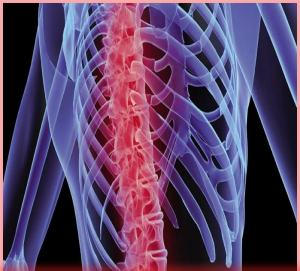
# Regenerative Medicine: The Promise

- **Potential to repair or replace cells and tissues** damaged by injury, disease, or aging
  - Restore normal structure and function
  - Enhance the body's innate healing capacity
- **Wide range of technologies**
  - Engineered biomaterials and tissues
  - Gene editing or replacement
- **Many potential applications**
  - Chronic diseases
  - Acute injury
  - Congenital defects



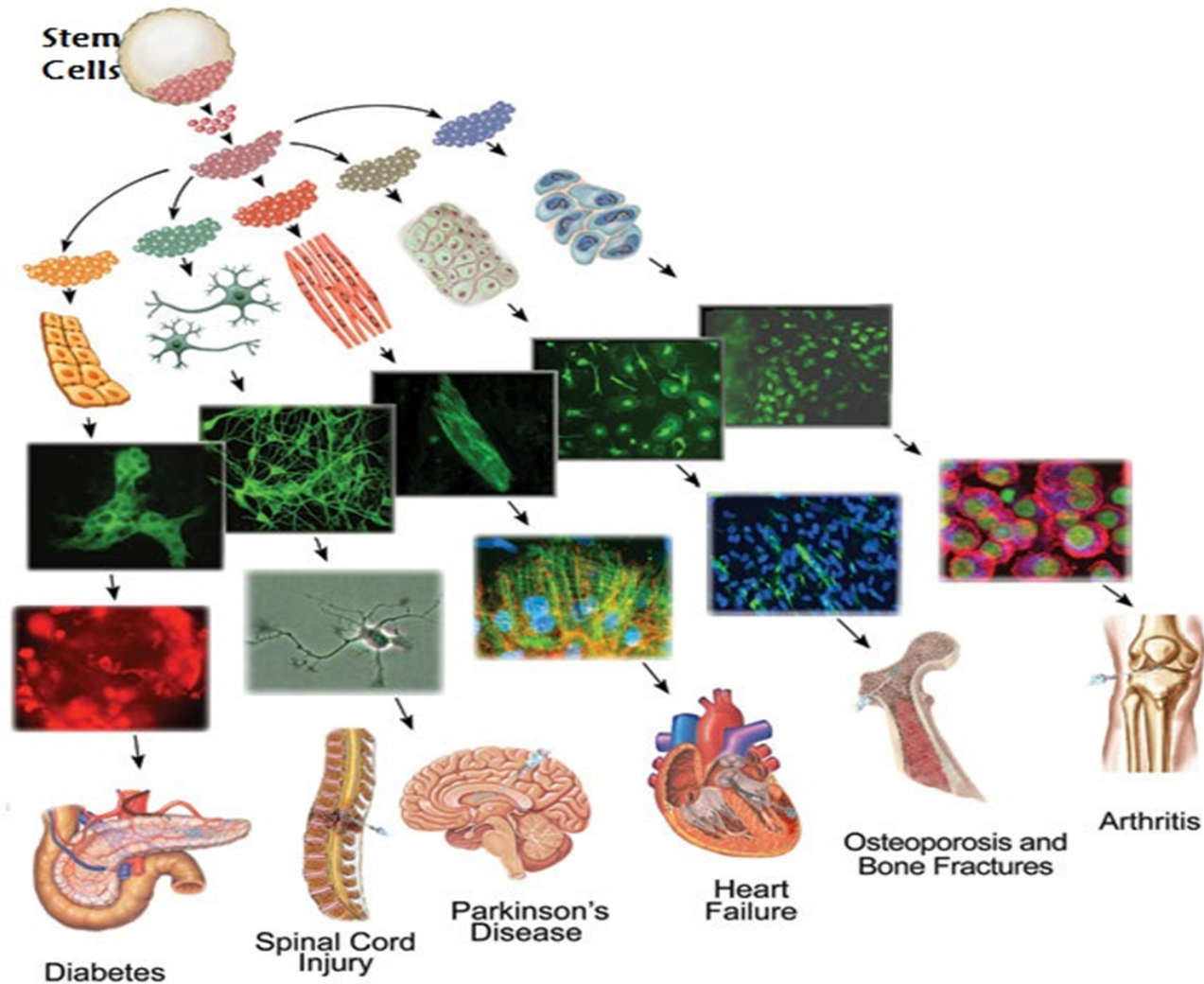
# Regenerative Medicine: Broad Applications Across Disease Areas

- Spinal cord injury
- Musculoskeletal injury and tissue degenerative conditions
- Autoimmune diseases
- Vision
- Neurodegenerative conditions
- Diabetes
- End-organ disease
- Hemoglobinopathies



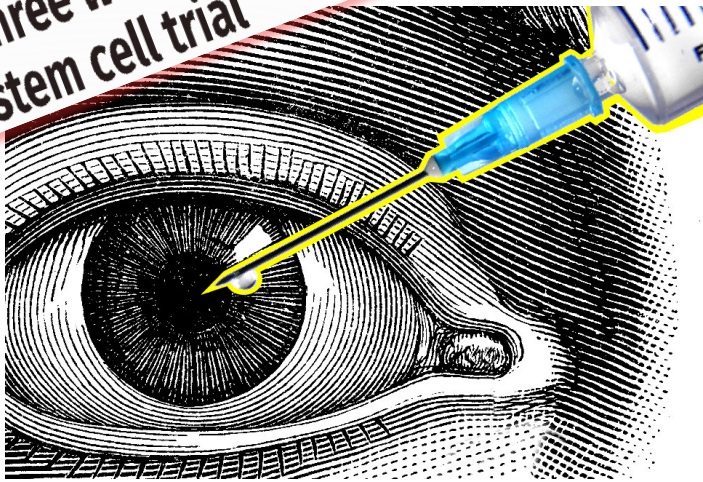
# Challenges

*Knowledge gaps, technical hurdles, and operational barriers*



# Challenges: Public Trust and Oversight

Three women 'left blinded after botched stem cell trial' INDEPENDENT



- **“Hype” in marketing, untested and unapproved RM products advertised**
  - Clinics advertising “NIH-registered trials”
  - “Patient-provided research payments” (\$8K-\$20K/protocol treatment)
  - A number of trials are not under IND
- **Need:**
  - Rigorous science
  - Regulatory oversight
  - National platform for discussion of the promise and challenges

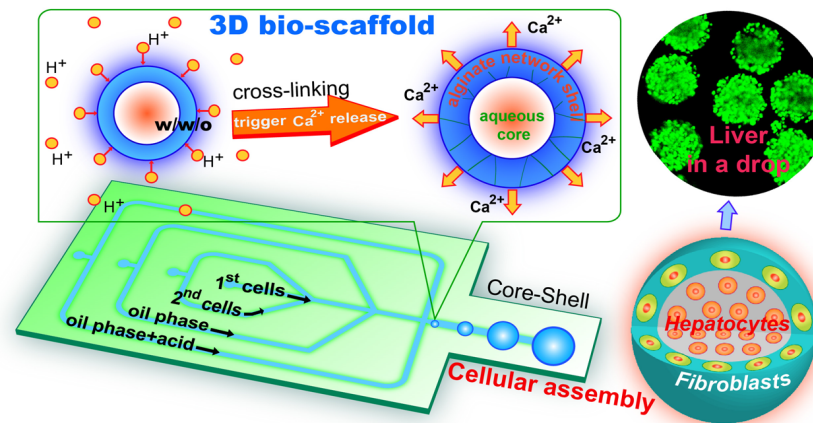
TRUST

# Innovation Project: Regenerative Medicine

## From the Act:

For the NIH, in **coordination with the FDA**, to award **grants and contracts for clinical research to further the field of regenerative medicine using adult stem cells, including autologous stem cells, ...not to exceed a total of \$30M, as follows:**

- FY 2017: \$2M
- FY 2018: \$10M
- FY 2019: \$10M
- FY 2020: \$8M



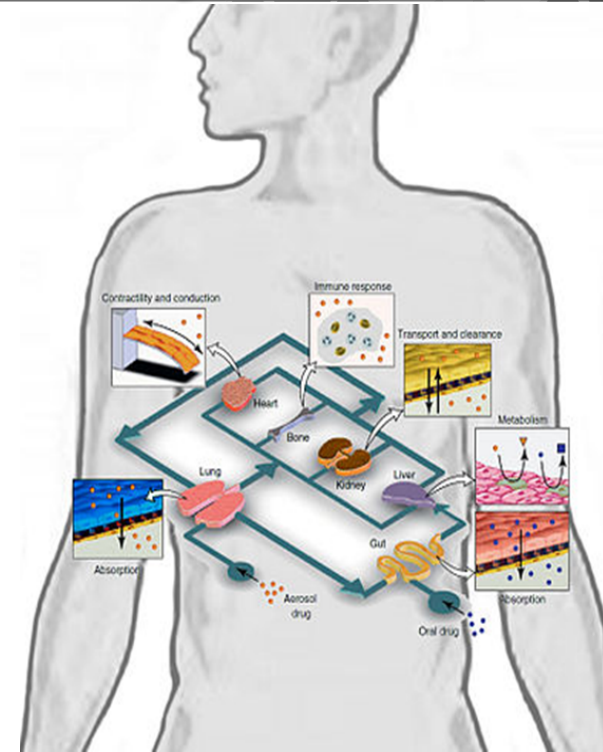
# Regenerative Medicine Funds: Matching Requirement

- **The Act further stipulates that:**
  - Awards must be contingent upon the recipient making available non-Federal contributions in an amount not less than \$1 for each \$1 of Federal funds provided in the award (i.e., **a matching requirement**)



# 21<sup>st</sup> Century Cures Act RM Provisions: Trans-NIH Collaborative Catalysts

- **Opportunity:**
  - Catalyze a focused and systematic trans-NIH approach to advancing the field
  - Work with the FDA to address patient safety, public trust, and the integrity of clinical research in RM
- **Modus Operandi:**
  - Consultative and inclusive
  - Leverage existing resources and infrastructure where possible





# RM Innovation Project Funds

Fiscal Year	Authorization (No Year \$)	Total Funds w/Matching	Description
2017	\$2 million	\$4 million	<ul style="list-style-type: none"> <li>• NOITP to alert community to new funding opportunities (<b>competitive supplements in FY17 and new projects in FY18-20</b>) and to matching requirements</li> <li>• Applications for supplemental funds to be solicited through a FOA that includes the matching funds requirement</li> <li>• Applications to be reviewed by NIH <b>in coordination with FDA</b>, followed by secondary <b>review by Council</b></li> </ul>
2018	\$10 million	\$20 million	Solicitation of projects to be <b>informed by expert consultation</b>
2019	\$10 million	\$20 million	Solicitation of projects to be informed by <b>workshop consultation</b>
2020	\$8 million	\$16 million	Solicitation of projects to be informed by <b>workshop consultation</b>

# RM Innovation Project Work Plan: Criteria

- **For FY17 funds, proposals will be assessed using standard NIH review criteria, as well as the following:**
  - Ability to put supplemental funding to good and immediate use
  - Underlying project is well along/already underway
  - Matching funds available at time of award
  - Contributes to breadth/diversity of RM science
  - Addresses a critical knowledge gap and/or technical barrier
  - Will help to significantly build or advance the field of RM
  - Contributes to regulatory science in RM



# ACD Feedback and Dialogue

- **What is your sense of the opportunities and challenges?**
- **What are priority research topics that we should focus on?**





National Heart, Lung,  
and Blood Institute