

# The 21<sup>st</sup> Century Cures Act

## Implementation of Regenerative Medicine Provisions

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**NIH Advisory Committee to the Director**  
**December 14, 2017**



# 21<sup>st</sup> Century Cures Act: RM Provisions

- **RM provisions applicable to NIH, FDA, and NIST**
- **Themes:**
  - Scientific rigor
  - Accelerating progress
  - Stimulating innovation and partnership
  - Appropriate regulatory oversight and standards



# 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



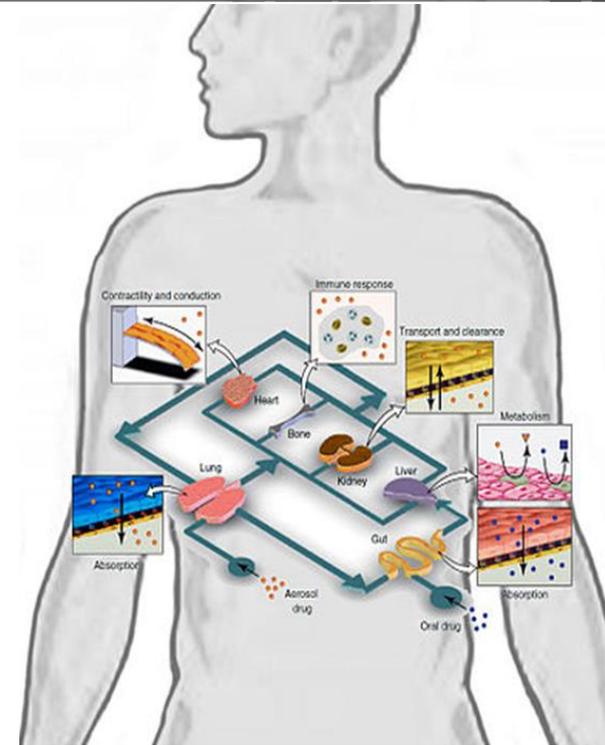
# Regenerative Medicine Funds: Matching Requirement

- **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**)



# RM Innovation Project: Opportunity to Catalyze Change

- **Opportunity:**
  - Catalyze a focused and systematic trans-NIH approach to advancing the field
  - Work with the FDA and research community to address patient safety, public trust, and the integrity of clinical research in RM
- **Modus Operandi:**
  - Consultative and inclusive
  - Stimulate new partnerships and development of platform technologies



# Partnership with FDA

- **Engagement of FDA Leadership:**
  - Preparation of a RMIP workplan requested by Congress
  - Established MOU for collaborations
  - Developing FOAs for FY17
  - Determination of whether FY17 applicants will need IND/IDEs
  - Planning and co-hosting of workshop on RM innovation with a focus on adult stem cells
  - Development of plans for FY18 and beyond



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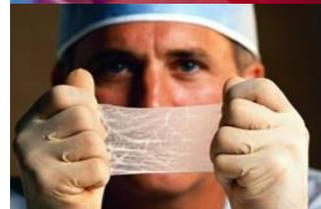
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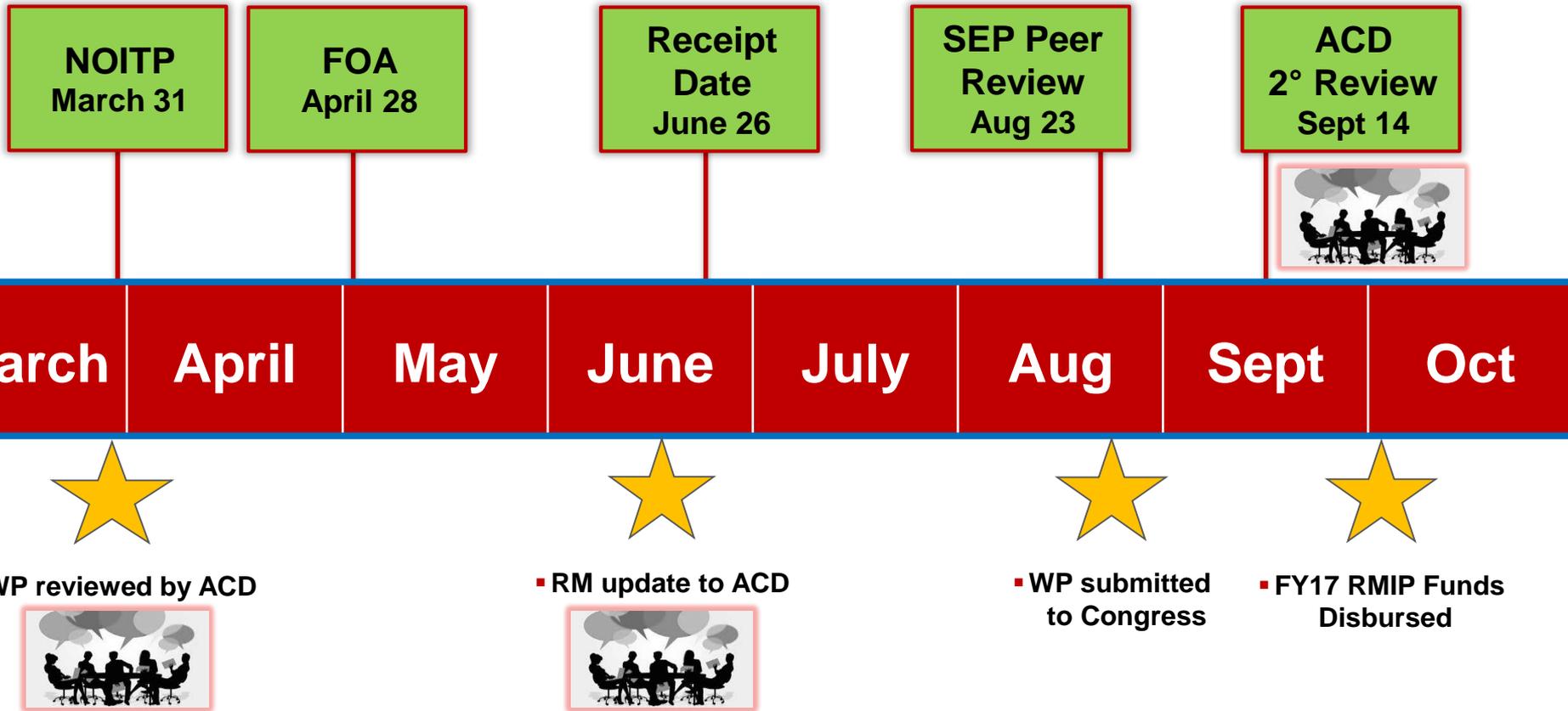
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# RMIP Implementation: FY 2017

- **Regular ACD Engagement**
- **Solicitation and Review of RMIP Proposals**
  - **NOITP and 12 FOAs were developed collaboratively by NIH and FDA for:**
    - **Cell-based approaches establishing evidence base for clinical applications including Pre-IDE/IND or IDE/IND**
    - **Development of Good Manufacturing Practices**
    - **Solutions to widely recognized challenges**
  - **Review conducted via a SEP and ACD**
- **\$2.7M awarded to support 8 competitive supplement proposals (\$0.7M from ICs)**



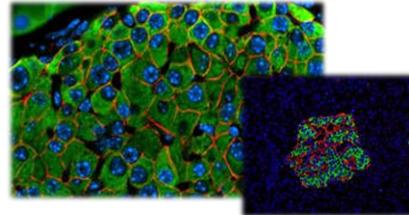
# Timeline of FY17 RMIP Events



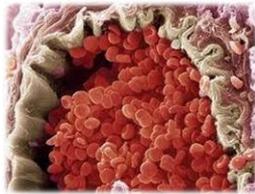
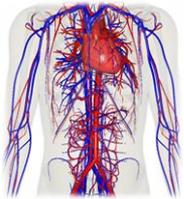
# Clinical Features of 8 Funded FY17 RMIP Applications



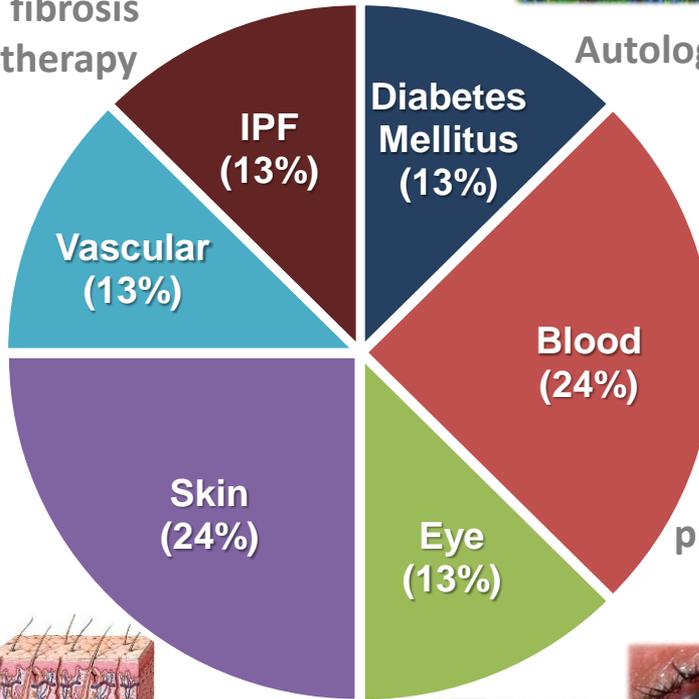
Idiopathic pulmonary fibrosis treatment using iPSC therapy



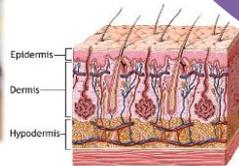
Autologous beta cell replacement therapy



Therapeutic revascularization by endothelial cell transplantation



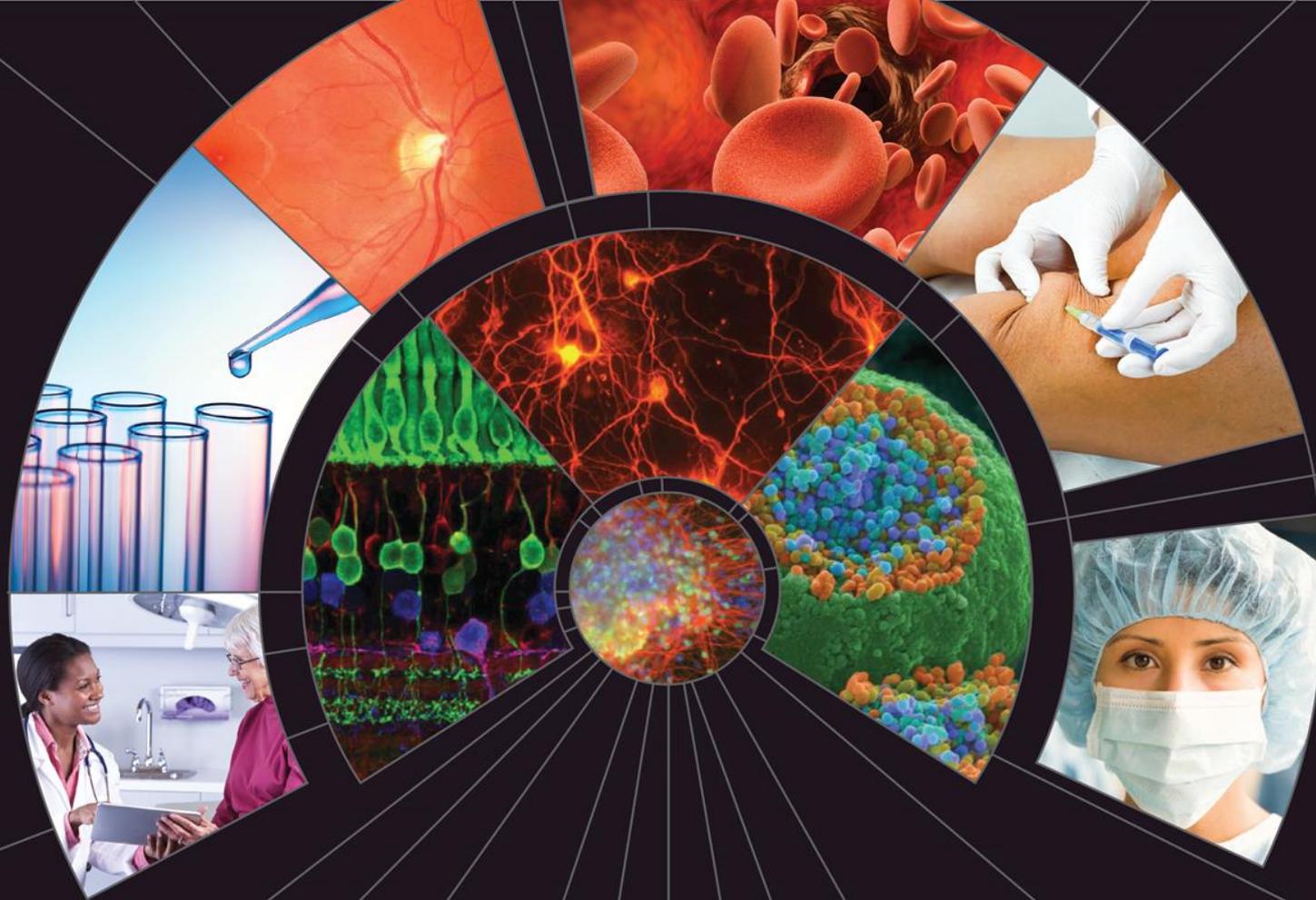
Engineered platelet production and optimization of RBC transfusions



Wound healing and epidermolysis bullosa therapy using iPSC targeting



Corneal engineering through LSC therapy



# Regenerative Medicine Innovation Workshop

Focus on Adult Stem Cells

December 6-7, 2017



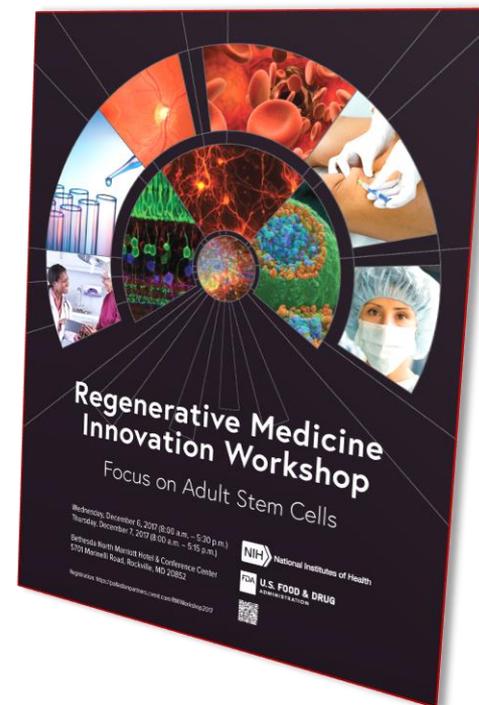
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# RM Innovation Workshop Overview

- **Opening Session**
  - Remarks by NIH Director and FDA Commissioner
  - Keynote address by Dr. Sally Temple (Neural Stem Cell Institute)
- **6 Sessions in Promising Clinical Areas**
  - Musculoskeletal Tissues and Integument
  - Endocrinology
  - Ophthalmology
  - Neurology
  - Hematology
  - Cardiology and Vascular Biology
- **2 Regulatory Science Sessions**
  - Stem cell-based product development
  - Clinical trial design
- **360° Panel: Multidisciplinary Discussion of Critical Gaps and Solutions**



# Funding Core Resources and Infrastructure



## RM Clinical Expertise

- Clinical trial network
- Clinical trial design resources
- GCPs

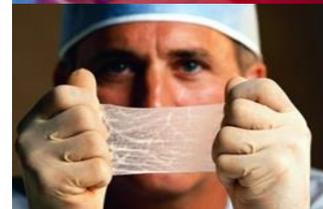
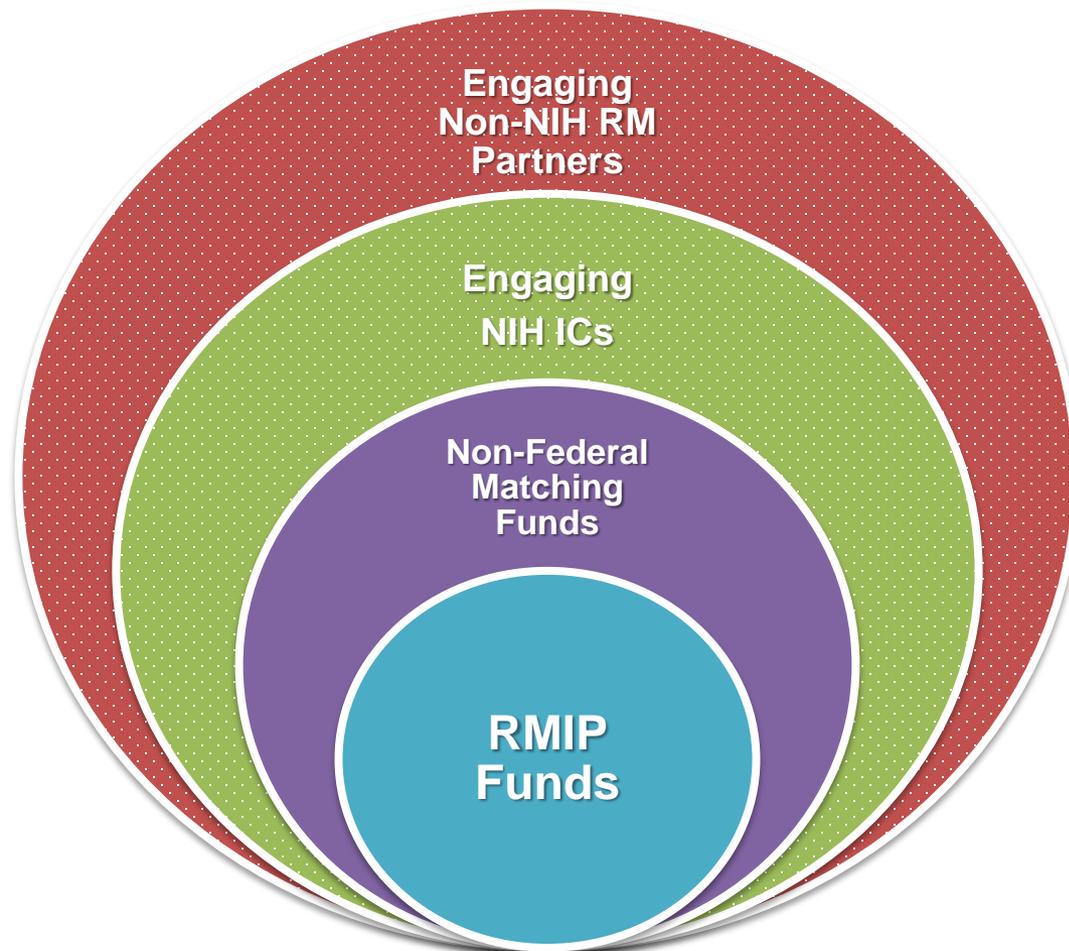
## RM Product Expertise

- Platform technologies for cell isolation and characterization
- Cell processing resources
- GMPs

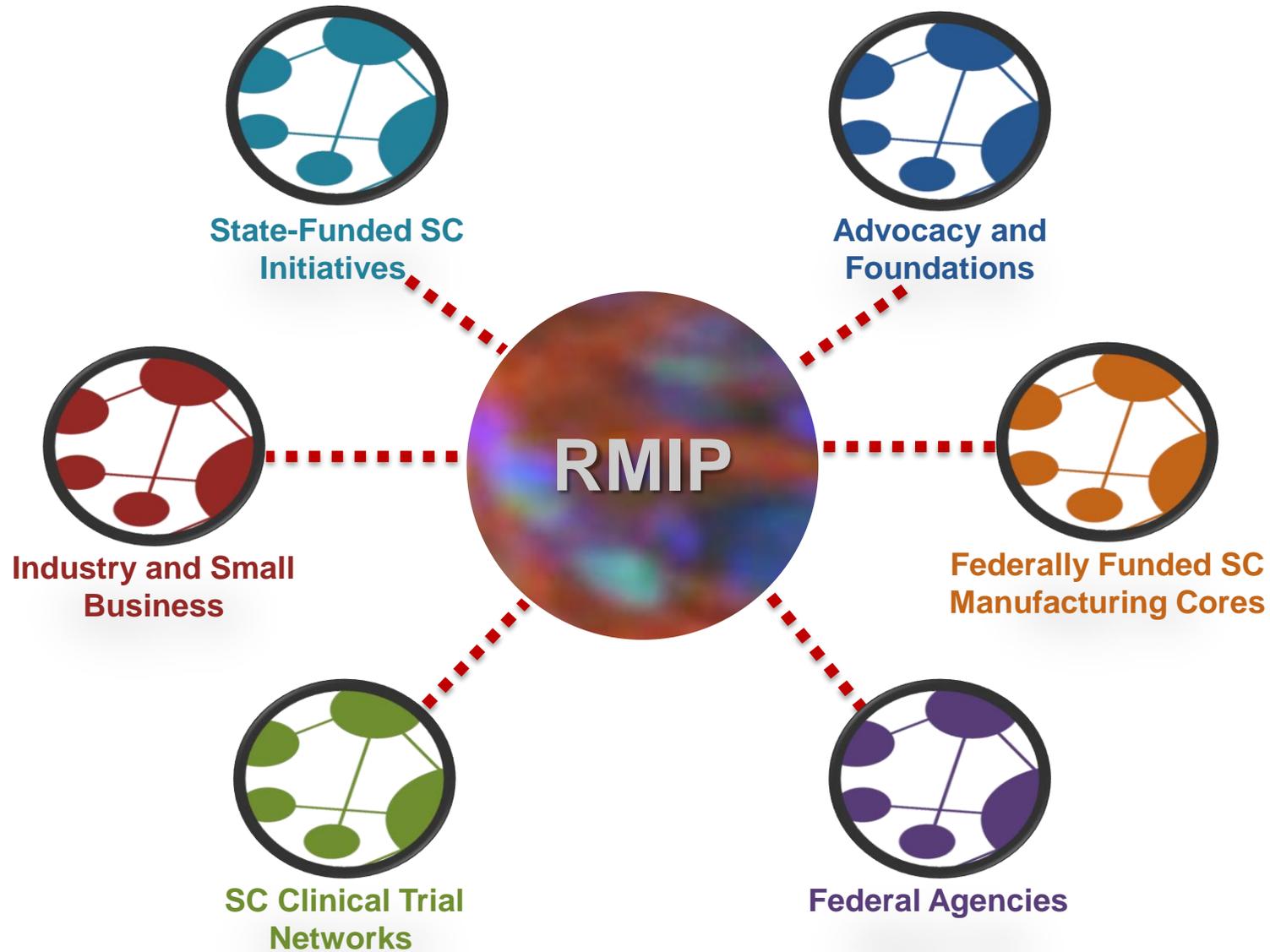
## RM Regulatory Expertise

- Regulatory core/framework

# RMIP Strategy FY18 and Beyond: Maximizing Impact

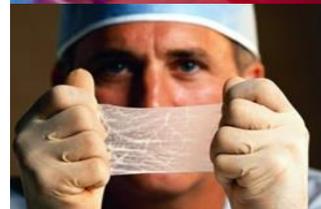


# Toward a RM Cooperative: Stakeholders and Potential Partners



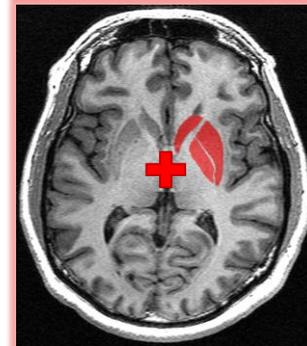
# Proposed Approach for FY18 and Beyond: Consultative and Collaborative

- **Fund best RM science with optimal stewardship**
  - Solicit investigator-initiated proposals
  - Implement a phased, milestone-driven approach to funding RM clinical trials
- **Create a diverse RM research portfolio**
  - Strategic focus on RM areas well-poised for clinical application
  - Studies that demonstrate proof-of-concept for clinical applications, including Pre-IND/IDE or IND/IDE
  - Studies that address widely recognized RM challenges
- **Forge key partnerships and collaborations**
  - Develop core resources and infrastructure critical to RM innovation



# Looking Ahead

- **The provision in the Cures Act for a Regenerative Medicine Innovation Project serves as a timely stimulus for NIH and FDA to:**
  - **Work together to galvanize the field**
  - **Foster major scientific advances**
  - **Address key regulatory and technical issues in product development and clinical investigation**
  - **Ensure that RM clinical studies are standardized, reproducible, and generalizable**





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