
Gary H. Gibbons, MD
Director, NHLBI

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
- Neurogenerative conditions
- Diabetes
- End-organ disease
- Hemoglobinopathies
Challenges

Knowledge gaps, technical hurdles, and operational barriers
Challenges: Public Trust and Oversight

- “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
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
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)
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

<table>
<thead>
<tr>
<th>Fiscal Year</th>
<th>Authorization (No Year $)</th>
<th>Total Funds w/Matching</th>
<th>Description</th>
</tr>
</thead>
</table>
| 2017        | $2 million               | $4 million             | - NOITP to alert community to new funding opportunities (competitive supplements in FY17 and new projects in FY18-20) and to matching requirements  
- Applications for supplemental funds to be solicited through a FOA that includes the matching funds requirement  
- Applications to be reviewed by NIH in coordination with FDA, followed by secondary review by Council |
| 2018        | $10 million              | $20 million            | Solicitation of projects to be informed by expert consultation |
| 2019        | $10 million              | $20 million            | Solicitation of projects to be informed by workshop consultation |
| 2020        | $8 million               | $16 million            | Solicitation of projects to be informed by workshop consultation |
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?