

# **Vision for the NIH and the Role of the Council of Councils**

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**Francis S. Collins, MD, PhD**

**NIH Director**

**November 16, 2009**



**My first 97 days as NIH Director.....**



# NIH: Steward of Medical and Behavioral Research for the Nation

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“Science in pursuit of **fundamental knowledge** about the nature and behavior of living systems... and the **application of that knowledge** to extend healthy life and reduce the burdens of illness and disability.”





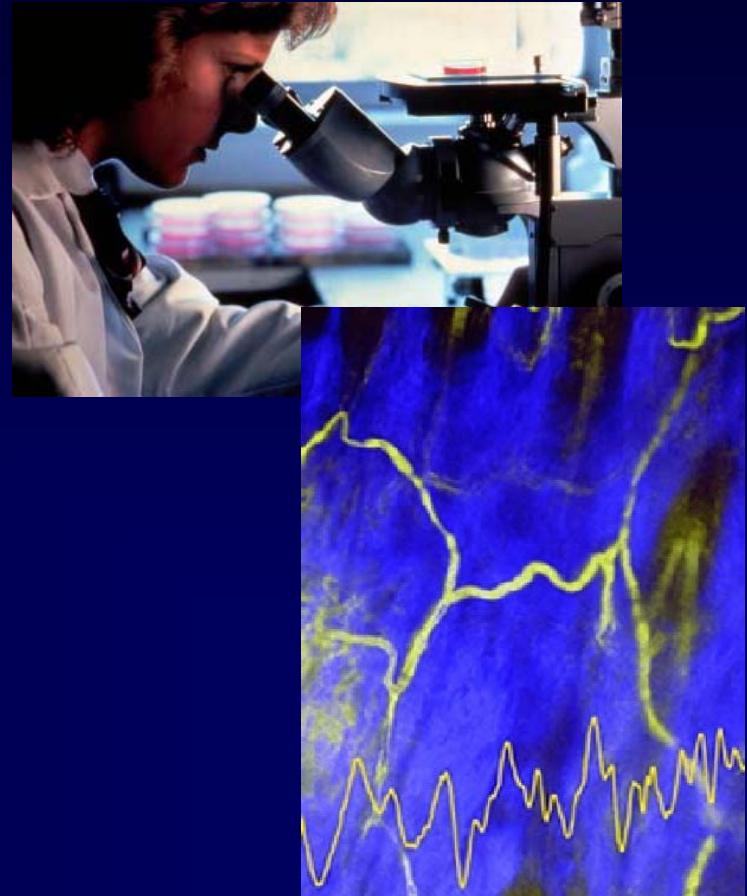
# Investigator-Initiated Research:

## *The Foundation of Biomedical Advances*

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**“... the hope of major advances lies in sustaining broad and free-ranging inquiry into all aspects of the phenomena of life, limited only by the criteria of excellence, the scientific importance, and the seriousness and competence of the investigator.”**

**– James A. Shannon, M.D.  
8th Director of NIH**



# Opportunities in Biomedical Research

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- Opportunity #1:  
Applying unprecedented opportunities in genomics and other high throughput technologies to understand fundamental biology, and to uncover the causes of specific diseases



# Opportunities in Biomedical Research

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- **NextGen DNA sequencing and beyond**
- **Nanotechnology**
- **Small molecule screening**
- **New imaging modalities**
- **Emphasis on comprehensive approaches**
- **Computational biology is critical**
- **Examples that are ripe for expanded effort**
  - Cancer
  - Autism
  - Microbiome
  - Many more....

# Opportunities in Biomedical Research

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- Opportunity #2:

Translating basic science discoveries into new and better treatments

- Moving from...



# Opportunities in Biomedical Research

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- To...





# Opportunities in Biomedical Research

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- **Stage set for NIH to play an expanded role in translation**
  - New discoveries about the fundamental basis of disease
  - Resources empowering academic investigators to develop lead compounds and “de-risk” projects
  - Opportunity for public-private partnerships
- **Small molecules**
  - Roadmap provides HTS capabilities that now exceed that of most pharmaceutical companies
  - Many ICs are ramping up investments
  - Therapeutics for Rare and Neglected Diseases (TRND) program now explicitly tackles the Valley of Death
  - Common diseases are increasingly seen as a collection of rare diseases, so they fit this model too
- **Stem cells (including hESCs and iPSCs)**
  - Explosion of new information likely with new NIH policy
  - Therapeutic uses uncertain but urgent to pursue

# Opportunities in Biomedical Research

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- Opportunity #3:  
Putting science to  
work for the benefit of  
health care reform



# Opportunities in Biomedical Research

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- **Comparative effectiveness research**
- **Prevention and personalized medicine**
  - Behavioral research
- **Health disparities research**
- **Pharmacogenomics**
- **Large scale prospective studies**
  - National Children's Study
  - Study of adult onset diseases?
- **Health IT**
- **Health economics research**

# Opportunities in Biomedical Research

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- Opportunity #4:  
Encouraging a  
greater focus on  
global health





# Opportunities in Biomedical Research

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- **Emphasis on global health fits with U.S. emphasis on “soft power”**
- **Scientific advances make an attack on infectious diseases more feasible than ever**
  - RNAi
  - Small molecule screening
  - Genomics
  - Vaccine development
- **Push beyond AIDS, malaria, TB to long list of neglected tropical diseases (NTDs) affecting hundreds of millions of people worldwide**
- **Include chronic non-communicable diseases and injuries, responsible for more 50% of deaths in developing world**

# Opportunities in Biomedical Research

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NIH Roadmap for Medical Research

- Opportunity #5:  
Reinvigorating and  
empowering the  
biomedical research  
community



# Opportunities in Biomedical Research

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- **Strive to avoid the post-ARRA cliff**
- **Reinvigorating the Common Fund**
- **Emphasis on innovation**
  - Pioneer awards
  - New Innovator awards
  - Transformative RO1s
- **Assessing results of current innovations in peer review**
- **Training programs**
  - Seek to reduce age at first grant award
  - Underrepresented minority programs
  - Work force analysis – what's the right answer?

# Advisory Committees

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- **Four key external committees advise the NIH director:**
  - **Advisory Committee to the NIH Director**  
*(NIH Director co-Chairs)*
  - **Council of Public Representatives**  
*(NIH Director co-Chairs)*
  - **Scientific Management Review Board**  
*(NIH Director is ex officio)*
  - **Council of Councils:** *Advises the NIH Director*



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**“A committee is a cul-de-sac  
down which ideas are lured and  
then quietly strangled.”**

**Sir Barnett Cocks  
(1907 -1989)**

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**“It is better to give than to  
receive – especially advice.”**

**Mark Twain  
American Author and Lecturer  
(1835 – 1910)**

# Advisory Committee to the NIH Director

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- **Focus:**
  - Program development
  - Resource allocation
  - NIH administrative regulation and policy
  - Other aspects of NIH policy
- **Specific Activity:**
  - Advises on grant applications and cooperative agreements for research and training



# Council of Public Representatives

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- **Focus:**
  - Public participation in NIH activities
  - Outreach and public communication efforts
  - Other matters of public interest
- **Specific Function:**
  - Provides a formal mechanism for public input into the research decision-making and priority-setting process





# Scientific Management Review Board

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- **Focus:**
  - Ensuring the optimal organizational structure and function for the fulfillment of NIH mission
- Recommendations are provided to NIH, HHS, and Congress



# Council of Councils

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- **Focus:**
  - Policies and activities of the Division of Program Coordination, Planning, and Strategic Initiatives
- **Specific Activities:**
  - Advise on research responsive to emerging scientific opportunities, public health challenges, and knowledge gaps
  - Advise the IC Directors during review process for trans-NIH initiatives

# Mandate for the Council of Councils

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- The Council was established by the NIH Reform Act of 2006.
- The Council's mandate is to advise me on DPCPSI policies and activities.

One Hundred Ninth Congress  
of the  
United States of America

AT THE SECOND SESSION

*Begun and held at the City of Washington on Tuesday,  
the third day of January, two thousand and six*

An Act

To amend title IV of the Public Health Service Act to revise and extend the authorities of the National Institutes of Health, and for other purposes.

*Be it enacted by the Senate and House of Representatives of  
the United States of America in Congress assembled,*

SECTION 1. SHORT TITLE.

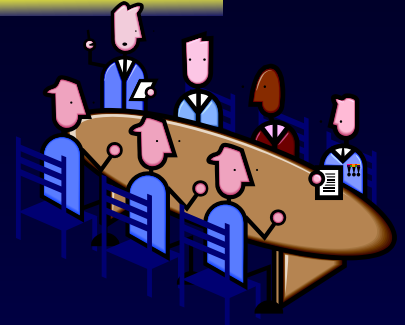
This Act may be cited as the "National Institutes of Health Reform Act of 2006".

TITLE I—NIH REFORM

# Role of the Council of Councils

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- Your job is to bring your knowledge of the ICs' missions and operations to the CoC -- not to serve as the official representatives from the IC Councils.
- May or may not report back to your IC Council on the efforts of this body.
- We need you to explore and provide advice beyond the research agenda of any one IC.





# Past Advice from the Council

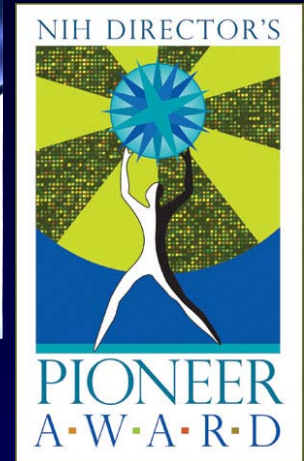
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- The Council has been asked to advise on:
  - The activities of the former OPASI
  - The activities of DPCPSI, such as RCDC system and the TR01 Program
  - Provide second-level review of the Transformative R01 grant applications
  - Early concept clearance of programs proposed for Common Fund support

# **The Common Fund in FY 2010: 13 programs covering the spectrum of the NIH mission**

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- **Clinical Research**
  - CTSAs
- **High Risk/High Reward**
  - Pioneers, New Innovators, TR01s
- **Resources to foster discovery and translation**
  - Molecular Libraries, Nanomedicine, ...
- **Interdisciplinary Research**
- **New**
  - Microbiome, Genotype/Tissue Expression Resources (GTEx), ...



# **Common Fund Forward: Demands and Needs**

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- **Ensure continuing support for existing Roadmap projects.**
- **Focus on future uses of the Common Fund.**
- **Common Fund commitment base is high – setting tight limits on new projects that can be initiated over next 2 years.**
- **Identify new ways for the Common Fund to be more facile.**
- **Select compelling projects for support.**

# Proposed New Efforts for Common Fund Support

- TRND/RAID expansion
- Regulatory/Translational Science
- Neglected Tropical Diseases/Global Health
- STEM Education
- Expansion of the Genotype-Tissue Expression Project
- Connectivity Map (LINCS)

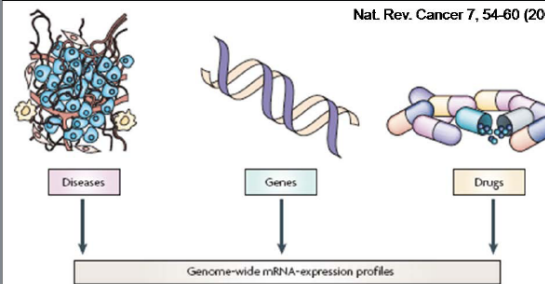


## The Connectivity Map: Using Gene-Expression Signatures to Connect Small Molecules, Genes, and Disease

Justin Lamb,<sup>1,\*</sup> Emily D. Crawford,<sup>1,†</sup> David Peck,<sup>1</sup> Joshua W. Modell,<sup>1</sup> Irene C. Blat,<sup>1</sup> Matthew J. Wrobel,<sup>1</sup> Jim Lerner,<sup>1</sup> Jean-Philippe Brunet,<sup>1</sup> Aravind Subramanian,<sup>1</sup> Kenneth N. Ross,<sup>1</sup> Michael Reich,<sup>1</sup> Haley Hieronymus,<sup>1,2</sup> Guo Wei,<sup>1,2</sup> Scott A. Armstrong,<sup>2,3</sup> Stephen J. Haggarty,<sup>1,4</sup> Paul A. Clemons,<sup>1</sup> Ru Wei,<sup>1</sup> Steven A. Carr,<sup>1</sup> Eric S. Lander,<sup>1,5,6</sup> Todd R. Golub<sup>1,2,3,5,7\*</sup>

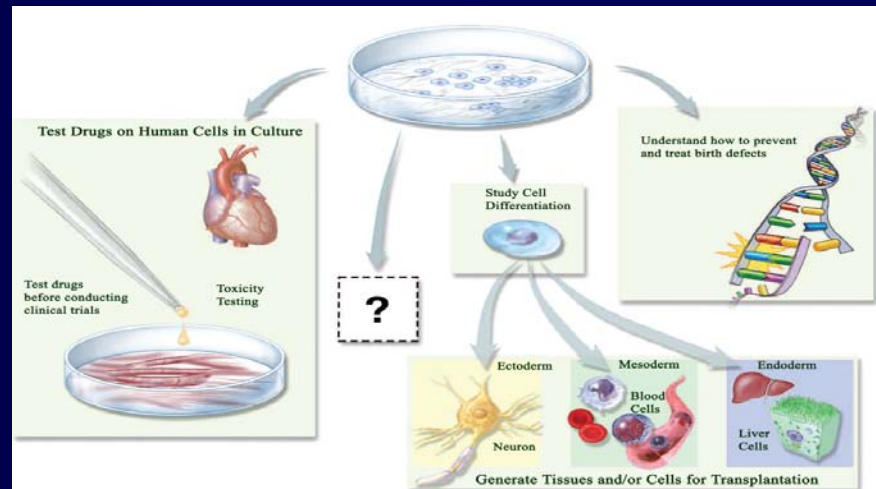
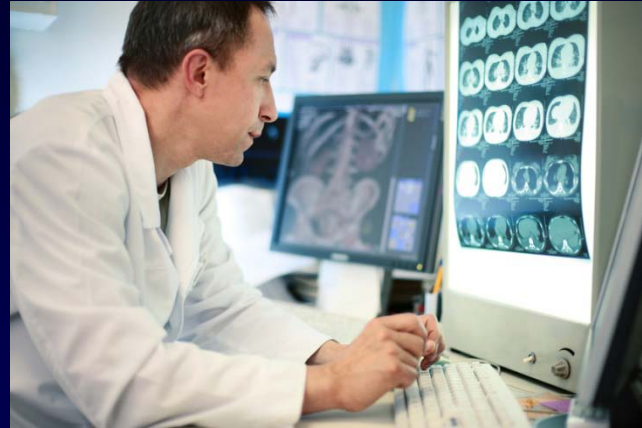
Science 313, 1929-1935 (2006)

Nat. Rev. Cancer 7, 54-60 (2007)



# Proposed New Efforts for Common Fund Support (continued)

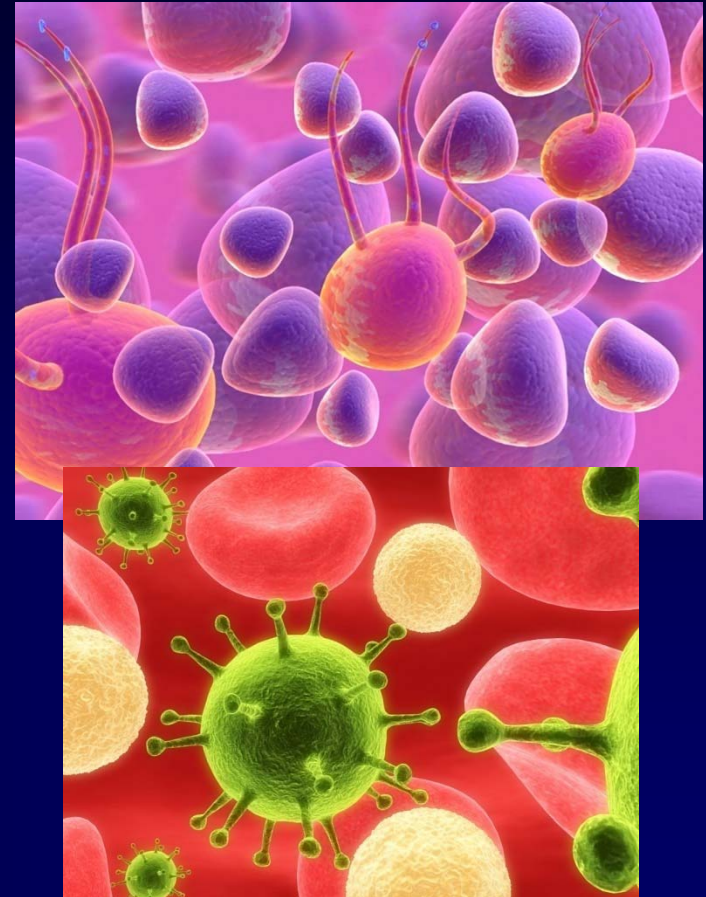
- Biobanks Workshop
- Health Economics Research
- Protein Affinity Reagents
- Mouse Phenotyping
- HMO Collaboratory
- Translational Stem Cell Research
- Science of Behavior Change



# Concept Proposals Previously Reviewed by the Council

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- We may pursue aspects of these proposals in the context of proposed new efforts
- LINCS and MAPGen seek to understand how gene networks operate in diverse cell types and how a single faulty network can underlie apparently distinct diseases
- Current consideration for a Connectivity Map aligns well with these goals – it aims to perturb networks and measure the cellular impact in a number of cell types.





# Concept Proposals Previously Reviewed by the Council

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- **An initiative similar to the Clinical Impact Awards**
- **Therapeutics for Rare and Neglected Diseases (TRND)**  
– is a new Trans-NIH program to facilitate drug discovery and development.



# Seeking Far-Reaching Common Fund Ideas

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- Should we convene brainstorming meetings with stakeholders, i.e., create “Grand Challenges” to design far-reaching efforts with the potential to transform health and medicine?
- Focus would need to be on projects for FY12 and beyond



# **Role of the Council of Councils**

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- **We will continue to seek your counsel on these efforts**
- **We welcome your ideas for research initiatives to gain new knowledge with the goal of improving health – here and around the world**
- **We are about to announce a search for a permanent DPCPSI Director**

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**Not failure, but low aim, is a crime.**

**— *James Russell Lowell***