



Exceptional Opportunities for Biomedical Research

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NIH Director

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My first 115 days as NIH Director.....



Presidential Visit to NIH – September 30, 2009



NIH: Steward of Medical and Behavioral Research for the Nation



“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.”

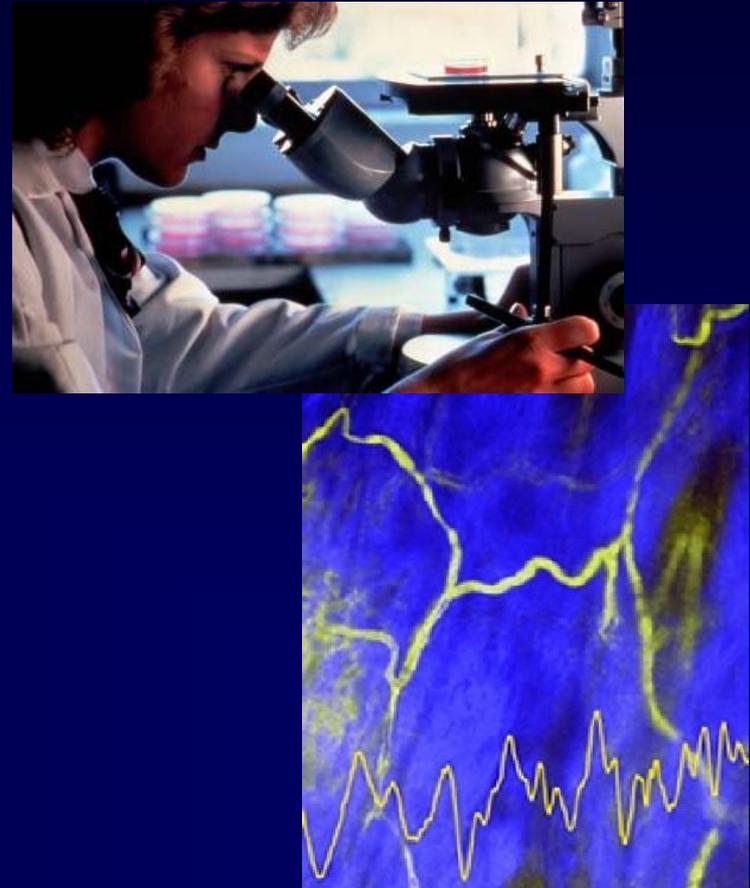


Investigator-Initiated Research:

The Foundation of Biomedical Advances

“... 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

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

- **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

- Opportunity #2:

Translating basic science discoveries into new and better treatments

- Moving from...



Opportunities in Biomedical Research

- To...



Opportunities in Biomedical Research

- **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 hESC and iPSC)**
 - Explosion of new information likely with new NIH policy
 - Therapeutic uses uncertain but urgent to pursue

Opportunities in Biomedical Research

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



Opportunities in Biomedical Research

- **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 research economics**
 - Going beyond clinical trials to studying health care delivery in the real world?
 - What payment incentives actually work to reduce costs and improve outcomes?

Opportunities in Biomedical Research

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



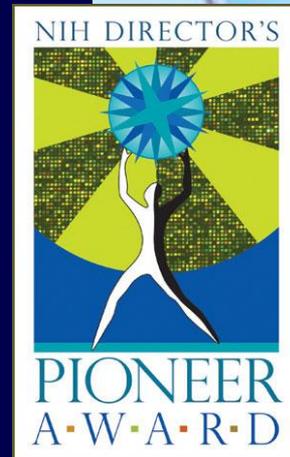
Opportunities in Biomedical Research

- **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 noncommunicable diseases and injuries, responsible for more 50% of deaths in developing world**

Opportunities in Biomedical Research



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



Opportunities in Biomedical Research

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

Advisory Committee to the NIH Director

- **First appointed:
November 1966**
- **Focus:**
 - Program development
 - Resource allocation
 - NIH administrative regulation and policy
 - Other aspects of NIH policy



The ACD has been an important advisory body

- **Provide perspective**
- **Provide feedback**
- **Provide expert advice**
- **A link between NIH and the biomedical research community**

Some ACD issues of discussion and deliberation:

- **Stem Cells**
- **Conflict of Interest**
- **Peer Review**
- **Regulatory Burden**
- **Priority Setting**
- **New Investigators**
- **Career Development**
- **Clinical Research**
- **Special Programs (e.g. Neuroscience Blueprint, Roadmap)**
- **Data Protection in Genome Wide Association Studies**
- **The American Recovery and Reinvestment Act**

Not failure, but low aim, is a crime.

— *James Russell Lowell*