



ROADMAP 1.5

**95th Meeting of the Advisory Committee to the Director
December 7, 2007**

**Alan M. Krensky, M.D., Director
Office of Portfolio Analysis and Strategic Initiatives
National Institutes of Health
Department of Health and Human Services**



Transforming Medicine: the NIH Roadmap

The NIH Roadmap is designed to foster the development of transformative solutions to grand challenges in health research

Addressing fundamental knowledge gaps

Providing infrastructure that supports clinical and translational research across the spectrum of NIH interests

Supporting investigators in new ways that encourage innovation, interdisciplinarity, and partnership



Transforming NIH: the NIH Roadmap

The NIH Roadmap represents a new way of doing business at the NIH

Funding cross-cutting research through a shared fund – the Common Fund

Collective planning and prioritization

Managing programs through inter-Institute teams; piloting new award mechanisms

Operating as a single entity for programs of shared interest



The Common Fund

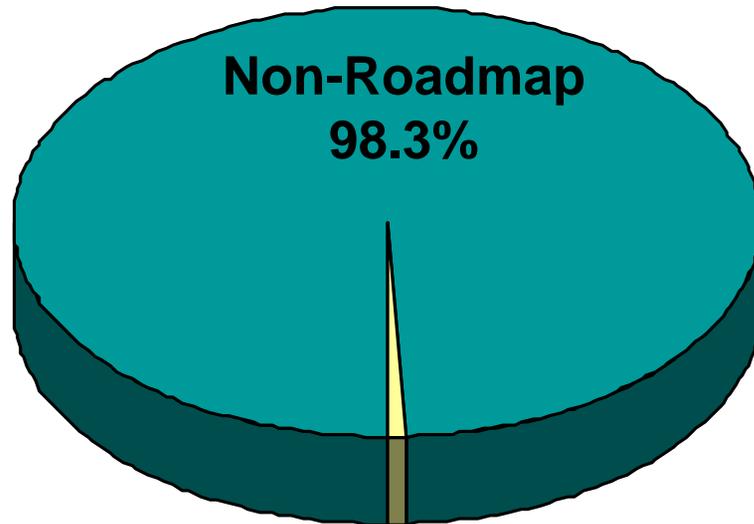
The NIH Reform Act of 2006:

1. Codifies Common Fund in PHS Act
2. No formula for growth established, but cannot drop as a percentage of the NIH Budget
3. Common Fund Strategic Planning Report required biennially
4. Review required when Common Fund reaches 5% of the NIH Budget



Roadmap for Medical Research

FY2007 Budget = \$28.9 B



Roadmap
1.7%: \$483M

¹ Award Rate differs from Success Rate as it includes all research grant mechanisms of support, incl. training awards

- Developed to increase synergy across NIH and to incubate new ideas
- Not a single initiative but 865 new awards
 - 716 investigators
 - 193 Institutions in USA
 - 41 states
 - Award rate¹ FY04 16.7%
FY05 18.2% FY06 19.5%
FY07 9.2%





Roadmap: Revolving Set of Programs to be Incubated

The NIH Roadmap, is, by design, a dynamic program intended to have revolving areas of emphasis.

It is designed to be an “incubator space” to pilot new programs, new funding mechanisms, to test new ways of approaching problems.

The first cohort of Roadmap initiatives will gradually transition out of the “incubator space” by fiscal year 2014.

New programs for the Roadmap will continue to be developed based on community input, cross-cutting relevance, and prioritization by the NIH Leadership.



The Roadmap and the Common Fund

Roadmap initiatives must demonstrate:

- 1) High potential to transform how biomedical and/or behavioral research will be conducted
- 2) Synergistic promotion and advancement of the individual missions of the ICs to benefit health
- 3) Applicability to issues beyond the scope of any one or small number of ICs
- 4) Likelihood that no other entity is able or likely to perform the work
- 5) A public health benefit of having the results of the research in the public domain.



How New Initiatives are Chosen

What

Scientific Consultation Meetings

NIH Staff Idea Submissions

Web-based Public Input/ Comment

When

Early '06

Summer '06

Fall '06

IC Directors

ACD

NIH Director

Decision to Fund Scientific Initiative

Who

Scientists from academia and industry, Council of Public Representatives

NIH Institute, Center, OD Program Office Director/Staff, Council of Public Representatives

Broad science and lay communities, Council of Public Representatives

Jan '07

May '07



Roadmap 1.5 update

- **Major Roadmap Programs**

Programs expected to consist of multiple, coordinated funding initiatives designed to overcome grand challenges in biomedical/health research. Each of these initiatives is expected to meet all of the criteria established for Roadmap Initiatives.

- **Pilot Studies**

Small, targeted proposals designed to test the potential for new technologies to revolutionize biomedical/health research.

- **Coordination Areas**

Cross cutting areas of critical importance to many ICs which receive significant support through diverse mechanisms and programs and which therefore require a coordinated effort for effective planning and management.

- **Strategic Planning Areas**

Broad, complex topics that are likely to require an extensive planning process to articulate grand challenges and solutions.





Grand Challenges Sort into Three Themes

Reengineering the Clinical Research Enterprise

Infrastructure, Tools, Services, Training Needed to Transform Clinical Research

New Pathways to Discovery

Basic Research, Infrastructure, Tools, Technologies needed to address fundamental knowledge gaps, to transform our ability to do research or our understanding of human health and disease

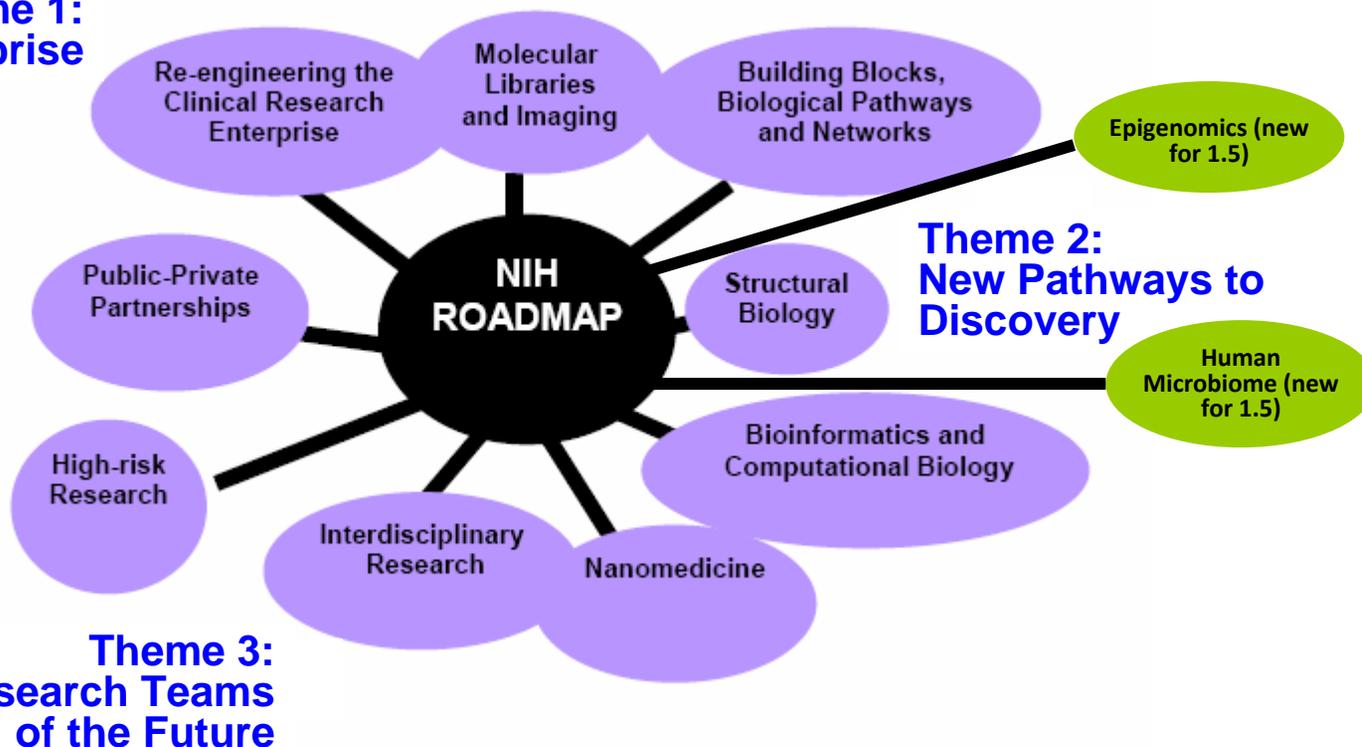
Research Teams of the Future

Tests new ways of supporting research to facilitate interdisciplinarity and partnership and to foster innovation



Three Key Themes of Roadmap for Medical Research

Theme 1: Clinical Enterprise





Roadmap 1.5 New Funding Opportunities

Epigenomics

- Technology Development in Epigenetics (R01)
- Technology Development in Epigenetics (R21)
- Epigenome Mapping Centers (U01)
- Epigenetic Data and Analysis Coordinating Center (U01)
- Discovery of Epigenetic Marks in Mammalian Cells (R01)
- Discovery of Epigenetic Marks in Mammalian Cells (R21)

Microbiome

- Reference Genomes and Metagenomic Survey (HM-1) (U54) -- limited competition
- Development of New Tools for Computational Analysis of HMP Data (HM-3-2) (R01), (R21)
- HMP related ELSI Studies (HM-6-1) (R01)
- Development of New Technologies Needed for Studying the Human Microbiome (R01), (R21)
- Establishment of a Data and Analysis Coordinating Center (DACC) for the HMP (U01)
- Demonstration Projects to relate disease to Changes in the Human Microbiome (HM-2) (UH2/UH3)



Epigenomics: the Next Step in Understanding the Human Genome

New Program for FY2008

The Epigenome is the totality of stable modifications to DNA that affect genetic activity without affecting the sequence of the DNA itself.

The Epigenome varies with cell type, reflecting cell type-specific genetic activity.

Changes in the Epigenome have been shown to correlate with some diseases, but the extent to which this is true is unknown.

The Roadmap will address fundamental challenges in epigenomic research to allow us to determine how the Epigenome contributes to human health and disease.



The Roadmap Epigenomics Program

The Roadmap Epigenomics Program will:

Develop comprehensive epigenome maps from many cell types;

Develop standardized platforms, procedures, and reagents for epigenomics research

Conduct demonstration projects to evaluate how epigenomes change in disease, with age, or following environmental exposures;

Develop new technologies for single cell epigenomic analysis and *in vivo* imaging of epigenetic activity; and

Create a public data resource to accelerate the application of epigenomics approaches.



Human Microbiome Project New Program for FY2008

What microbes live in humans?

How do they contribute to health? To disease?

Might the microbiota be manipulated to improve health?

The human body contains ten times as many microbial cells—bacteria and other micro-organisms—as it does human cells. These microbes, which are found in locations throughout the body, are thought to have a profound influence on many biological processes, including development, immunity, and nutrition. However, technical difficulties in isolating and studying many of these organisms have limited our ability to fully understand the effects of the microbiome on human health and disease.



Human Microbiome Project

The Human Microbiome Project will generate resources and support the development of new technologies and computational approaches to facilitate the characterization of the highly complex human microbiome.

This project will improve our knowledge of how changes in the microbiome correlate with changes in human health.



Roadmap 1.5 Upcoming Funding from FY08-FY12 (in millions)

Epigenomics (*TOTAL \$150.5 Million)

Technology Development in Epigenetics (R01) (R21)	\$21
Epigenome Mapping Centers (U01)	\$50
Epigenetic Data and Analysis Coordinating Center (U01)	\$7.5
Discovery of Novel Epigenetic Marks in Mammalian Cells (R01) (R21)	\$17.5
Epigenomics in Human Health and Disease	\$40

Microbiome (*TOTAL \$112.8 Million)

Reference Genomes and Metagenomic Survey (HM-1) (U54) - Limited	\$30.5
HMP related ELSI Studies (HM-6-1) (R01)	\$1.5
Development of New tools for Computational Analysis of HMP Data (HM-3-2) (R01), (R21) Development of New Technologies Needed for Studying the Human Microbiome (R01), (R21)	\$35.5
Establishment of a Data and Analysis Coordinating Center (DACC) for the HMP (U01)	\$11.3
Demonstration Projects to relate disease to Changes in the Human Microbiome (HM-2) (UH2/UH3)	\$28.3

* Total represents funding initiatives plus related workshops and research support.





Roadmap: the Next Cohort

A number of potential Roadmap programs were highlighted through last year's idea-gathering process, that may be developed into Roadmap programs in the future:

Human Phenotyping Program

Protein Capture/Proteome Tools

Connectivity Map

Strategic Planning in other areas may also produce Roadmap initiatives:

Health Disparities

Training/Workforce

The NIH will re-solicit ideas from the community on a regular basis.

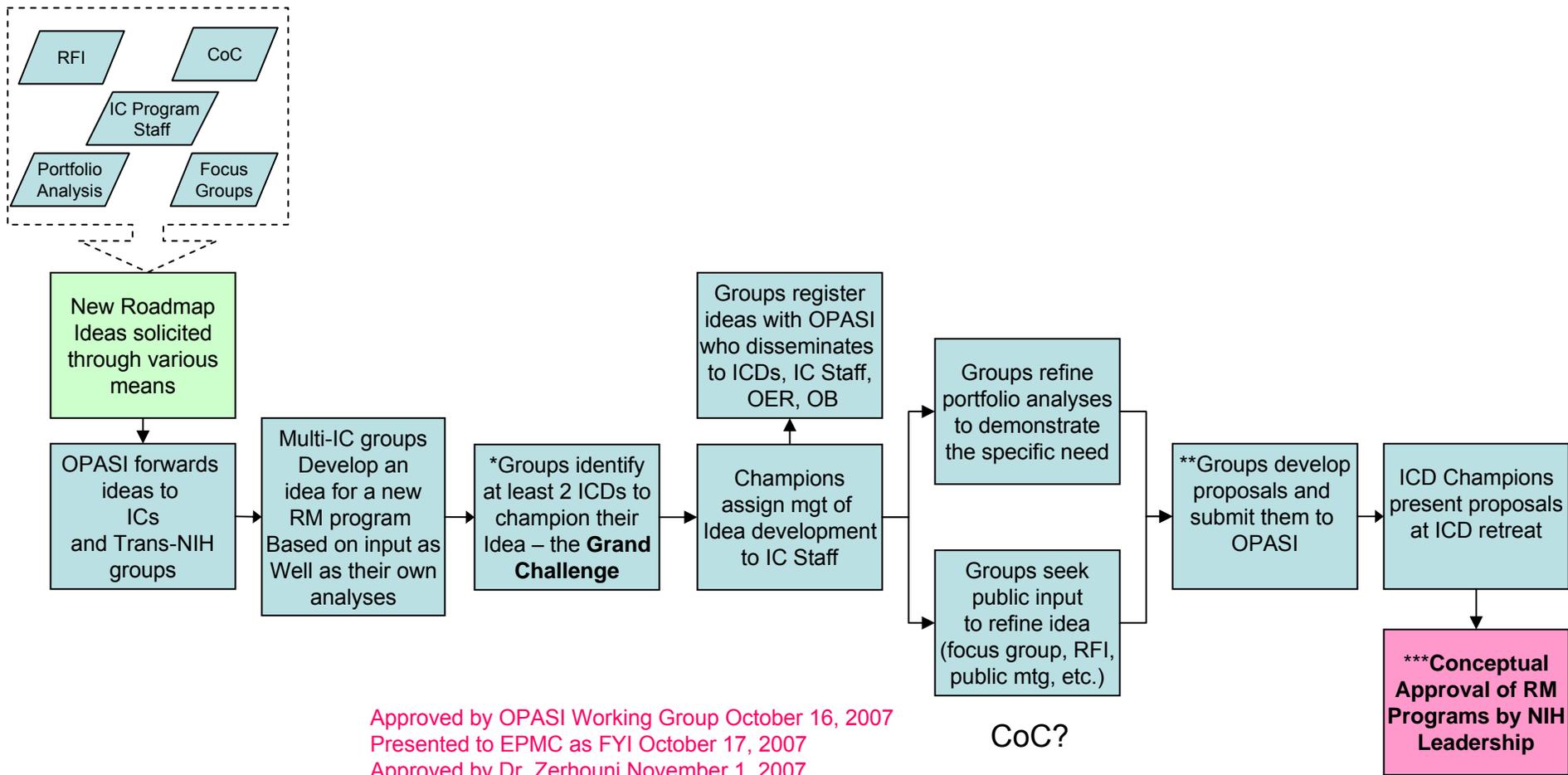


Demonstration Programs

Bridging the Sciences: grants for biomedical research at the interface between the biological, behavioral, and social sciences and the physical, chemical, mathematical, and computational sciences.

High-Risk, High-Reward: grants, contracts or “other transactions” for high-impact, cutting-edge research that fosters scientific creativity and increases fundamental biological understanding leading to the prevention, diagnosis, and treatment of diseases and disorders.

Selection of Roadmap Concepts Annual Process



NOTES:

* ICDs should feel sufficiently enthusiastic that they are willing to support the proposal at the NIH Leadership retreat in February. **They are not expected to personally lead and implement each Roadmap program; Program Staff may be delegated this role.**

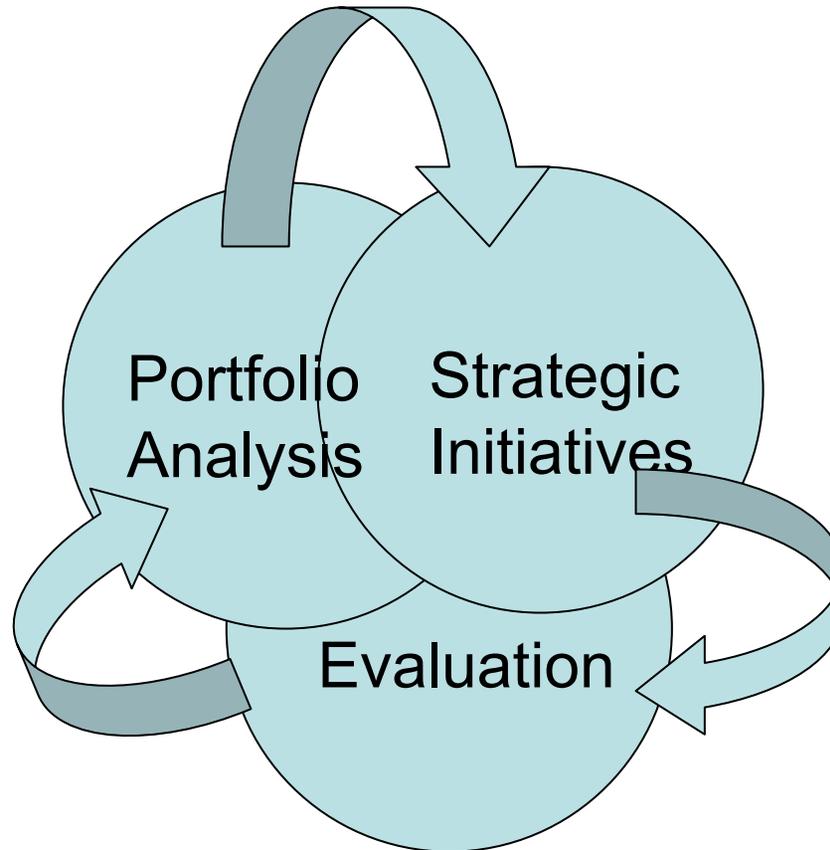
** The Proposal should include 1) Statement of “grand challenge” that the program is designed to address; 2) Proposed Initiatives that address the challenge; 3) Synopsis of public input and portfolio analysis.

*** Concept approval may include a directive that the concept come back to ICDs in a more refined form before final approval. RFAs for Summer release would go to the following May council for payment, and RFAs for Fall release would go to the following September council for payment in the next fiscal year.





OPASI STRUCTURE-FUNCTION





Discussion

1. The on-ramp for new ideas
2. The exit ramp (transitions)
3. Boundaries

