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## **NIH Roadmap Overview**

### **September 2003**

#### ***History and Purpose***

Soon after becoming the Director of the National Institutes of Health (NIH), in May 2002, Elias A. Zerhouni, M.D. convened a series of meetings to chart a "roadmap" for medical research in the 21<sup>st</sup> century. The purpose was to identify major opportunities and gaps in biomedical research that no single institute at NIH could tackle alone but that the agency as a whole must address to make the biggest impact on the progress of medical research. The opportunities for discoveries have never been greater, but the complexity of biology remains a daunting challenge. NIH is uniquely positioned to catalyze changes that must be made to transform our new scientific knowledge into tangible benefits for people

Developed with input from meetings with more than 300 nationally recognized leaders in academia, industry, government, and the public, the NIH Roadmap provides a framework of the priorities the NIH as a whole must address in order to optimize its entire research portfolio. It lays out a vision for a more efficient and productive system of medical research. It identifies the most compelling opportunities in three main areas: new pathways to discovery, research teams of the future, and re-engineering the clinical research enterprise.

Initiatives under the NIH Roadmap will help enable the agency to sustain its historic record of cutting-edge contributions that are central to extending the quality of healthy life for people in this country and around the world.

#### ***Steps in the Process***

The process of crafting the Roadmap – from vision to implementation – is described in the following sections.

The first step in the Roadmap process involved a series of five meetings in which Dr. Zerhouni and Directors of the various NIH Institutes led invited participants through lively discussions about the most compelling initiatives that the NIH should pursue over the next 10 years that will have the most profound impact on the progress of medical research, both in the United States and worldwide. Participants were asked:

- What are today's scientific challenges?
- What are the roadblocks to progress?
- What do we need to do to overcome roadblocks?
- What can't be accomplished by any single Institute – but is the responsibility of NIH as a whole?

During each meeting, participants were asked to step into the NIH Director's role and to prioritize different research areas.

### NIH Leadership Forum Meets to Define Action Plan

The priority areas identified through the Roadmap meetings formed the basis for the discussions at the 2002 NIH Leadership Forum – an annual retreat for NIH Institute and Center Directors. The Forum participants were organized into five groups to address the major themes that emerged from the roadmap meetings. Dr. Zerhouni charged the groups with critically assessing the input from the roadmap meetings – What can be done? What can't be done? What needs to be done? When can it be done? What is realistic?

In addition, Dr. Zerhouni asked the groups to consider compelling arguments for each proposed initiative and to assess the impact, feasibility, appeal to a wide constituency, and potential for real advances in medical research. Dr. Zerhouni stressed that he was not looking for "business as usual under another name." Instead, the groups should come up with exciting, enabling ideas and actions that can be clearly articulated to a wide audience. The groups identified short and long-term activities and actions; other activities that should be addressed in the future; and areas of science hindered by specific roadblocks. At the end of the day, each group had identified 3-5 major, trans-NIH themes for further consideration.

### Working Groups Develop Initial Blueprints for Action

In the months after the Forum, the new ideas were further refined. The development of proposed Roadmap initiatives required systematic analysis and planning. In the spring of 2003, a series of Institute Director-chaired Working Groups of NIH staff, along with ad hoc outside advisors, were formed. Thus, the action plans developed by the Working Groups served as the initial blueprints for building the medical research enterprise of tomorrow.

Each working group presented their top initiatives at the 2003 NIH Budget Retreat, attended by the NIH Director and Institute and Center Directors. The group examined the initiatives and weighed them in the context of several broad criteria:

- Is the initiative truly transforming – will it dramatically change how or what biomedical research is conducted in the next decade?
- Would the outcomes from the initiative be used by and synergize the work of many NIH Institutes and Centers?
- Can the NIH afford NOT to do it?
- Will the initiative be compelling to our stakeholders, especially the public?
- Does the initiative position the NIH as unique – doing something that no other entity can or will do?

### Implementation Groups

The Roadmap working groups were grouped into nine Implementation Groups. These nine groups devised implementation plans for the next stage of the Roadmap. These plans included timelines, milestones, mechanisms for coordination, need for inventories, staffing needed for program implementation.

### *Major NIH Roadmap Themes*

The NIH Roadmap is an integrated vision to deepen our understanding of biology, stimulate interdisciplinary research teams, and reshape clinical research to accelerate medical discovery and improve people's health. Most of the initiatives will begin in FY 2004. Other initiatives will start in FY 2005 and beyond, depending upon the budget and other emerging needs. The three NIH Roadmap themes are as follows:

#### New Pathways to Discovery

This theme of the NIH Roadmap addresses the need to advance our understanding of the daunting complexity of biological systems. Future progress in medicine will require a quantitative understanding of the many interconnected networks of molecules that comprise our cells and tissues, their interactions, and regulation. We need to more precisely know the combination of molecular events that lead to disease if we hope to truly revolutionize medicine. New Pathways to Discovery also sets out to build a better "toolbox" for medical research in the 21<sup>st</sup> century.

To fully capitalize on the recent completion of the human genome sequence and many recent discoveries in molecular and cell biology, the research community needs wide access to technologies, databases and other scientific resources that are more sensitive, more robust and more easily adaptable to researchers' individual needs. Among the resources to be established are libraries of chemical molecules that may provide: probes of biological networks; imaging probes for molecular and cellular events; improved computational infrastructure for biomedical research; nanotechnology devices capable of viewing and interacting with basic life processes; and potential targets for new therapies.

These initiatives will provide a solid scientific foundation for new strategies for diagnosing, treating, and preventing disease. Implementation groups in this area are:

- Building Blocks, Biological Pathways, and Networks
- Molecular Libraries & Molecular Imaging
- Structural Biology
- Bioinformatics and Computational Biology
- Nanomedicine

### Research Teams of the Future

The scale and complexity of today's biomedical research problems increasingly demands that scientists move beyond the confines of their own discipline and explore new organizational models for team science. For example, imaging research often requires radiologists, physicists, cell biologists, and computer programmers to work together on integrated teams. Many scientists will continue to pursue individual research projects; however, they will be encouraged to make changes in the way they approach the scientific enterprise. NIH wants to stimulate new ways of combining skills and disciplines in both the physical and biological sciences. The Director's Innovator Award will encourage investigators to take on creative, unexplored avenues of research that carry a relatively high potential for failure, but also possess a greater chance for truly groundbreaking discoveries. In addition, novel partnerships, such as those between the public and private sectors, will be encouraged to accelerate the movement of scientific discoveries from the bench to the bedside.

As part of its theme, Research Teams of the Future, the NIH Roadmap seeks to encourage scientists and scientific institutions to test alternative models for conducting research. Implementation groups in this area are:

- Interdisciplinary Research
- High-Risk Research - Director's Innovator Award
- Public-Private Partnerships

### Re-engineering the Clinical Research Enterprise

Ideally, basic research discoveries are quickly transformed into drugs, treatments or methods for prevention. Such translation lies at the very heart of NIH's mission. Although NIH has been historically successful by funding medical research that has helped to transform once acute and lethal diseases into more chronic ones, it has become clear to the scientific community that our country will need to recast its entire system of clinical research if we are to remain as successful as in the past.

Over the years, clinical research that helps discover mechanisms of disease, prevention, diagnosis, or treatment has become more difficult to conduct. Yet the exciting discoveries we are currently making require us to conduct even more efficiently the complex clinical studies required to make rapid medical progress and to further inform our basic science efforts. This is undoubtedly the most challenging, but critically important, area identified through the NIH roadmap process.

At the core of this vision is the need to develop new partnerships of research with organized patient communities, community-based physicians, and academic researchers. This also includes the need to build better integrated networks of academic centers linked to a qualified body of community-based physicians who care for sufficiently large groups of patients interested in working with researchers to quickly develop and test new interventions. This vision will require

new paradigms in how clinical research information is recorded, new standards for clinical research protocols, modern information technology platforms for research, new models of cooperation between NIH and patient advocates, and new strategies to re-energize our clinical research workforce.

Re-engineering the Clinical Research Enterprise is intended to address these pressing needs by promoting the better integration of existing clinical research networks, encouraging the development of technologies to improve the assessment of clinical outcomes, harmonizing regulatory processes, and enhancing training for clinical researchers. A major goal of this initiative is to more fully involve and empower the public in the research process.

Implementation groups in this area are:

- Harmonization of Clinical Research Regulatory Processes
- Integration of Clinical Research Networks
- Clinical Research Informatics: National Electronic Clinical Trials and Research System (NECTAR)
- Regional Translation Research Centers
- Enabling Technologies for Improved Assessment of Clinical Outcomes
- Enhance Clinical Research Training in the Medical Scientist Training Program and Multidisciplinary Training
- Create a National Clinical Research Corps

Taken together, the components of these initiatives are part of a well-thought out national portfolio of research to meet the health demands of the 21<sup>st</sup> century.

More information about the NIH Roadmap can be found at: <http://nihroadmap.nih.gov>. Further information about the NIH can be found at its Web site: [www.nih.gov](http://www.nih.gov).

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