

Introduction

Strategic Planning and Roadmap 1.5

Strategic planning at NIH takes place at many levels. The U.S. Congress, through the NIH authorization and appropriations processes, sets IC funding levels, establishes the missions for some ICs, and directs NIH attention to particular areas of research interest or emphasis⁵. The Administration also establishes priorities for improving the health of the Nation that must be addressed by NIH. An example is Healthy People 2010⁶, a comprehensive set of disease prevention and health promotion objectives for the Nation to achieve by 2010. [Healthy People 2010](#) has two overarching goals—“Increase Quality and Years of Healthy Life” and “Eliminate Health Disparities”—and NIH is understandably the lead or co-lead for many of the specific topic areas. In addition, NIH establishes its goals and priorities fully cognizant of the framework of the *HHS Strategic Plan Goals and Objectives - FY 2007-2012*⁷, which sets the stage for individual performance plans and outcome measures across NIH.

Some strategic plans pertain to the whole agency, for example, the NIH Roadmap for Medical Research. NIH initiated the Roadmap planning process in 2002 by consulting broadly with stakeholders to identify and prioritize the most pressing problems (roadblocks) facing medical research that could be uniquely addressed by NIH as a whole. Ideas for Roadmap initiatives were formulated from those initial consultations and then vetted based on whether the initiative had high potential to transform the way health research is conducted, would synergize with but cut across the individual missions of the ICs, would not be redundant with activities conducted by other agencies or entities, and be expected to have an impact on public health such that results should be broadly disseminated and in the public domain. This novel NIH-wide planning process launched a set of over 30 initiatives under three broad themes in 2003. The first set of Roadmap initiatives already is deepening our understanding of molecular biology and its role in health and disease; creating tools for 21st century biomedical research; stimulating interdisciplinary research teams; promoting high-risk breakthrough science; and reengineering the clinical research enterprise. NIH institutionalized this NIH-wide planning process when it established the Office of Portfolio Analysis and Strategic Initiatives (OPASI) in spring 2006. That summer, OPASI began soliciting ideas for the next generation of Roadmap initiatives. When NIH established OPASI, it also enhanced NIH's systems for gathering and analyzing information in support of strategic planning (see sections on *OPASI* and on “*Roadmap 1.5*, below).

Although the Roadmap process is novel, NIH has a significant tradition of NIH-wide and trans-NIH strategic planning. The *NIH Strategic Research Plan and Budget to Reduce and Ultimately Eliminate Health Disparities* is a prominent example of an NIH-wide plan. Trans-NIH strategic plans focus on areas that are best addressed by involving multiple ICs in identifying research goals and priorities; for example, the Strategic Plan for NIH Obesity Research was developed by the NIH Obesity Research Task Force, led by NIDDK and NHLBI. The Plan seeks to maximize collaboration among the ICs and OD Offices to capitalize on their respective capabilities. Recent initiatives (FY 2006 and 2007) relate to translational research for the prevention and control of diabetes and obesity (NIDDK and OBSSR); bioengineering and obesity (NHLBI, NCI, NIA, NIBIB, and NIDDK); and identifying and reducing factors within health care systems that result in disparate health outcomes for patients with diabetes or obesity-related conditions (NIDDK), among others. Other trans-NIH research plans address goals and objectives in areas that include neuroscience research, HIV/AIDS, liver disease, diabetes, health disparities, muscular dystrophies, autoimmune diseases, and more.

Naturally, however, the majority of strategic planning at NIH is IC-based. IC strategic plans function as guideposts to the investigative and NIH communities. Each NIH IC has unique processes for developing and disseminating its strategic plans, but by developing and articulating consensus on today's most pressing health needs and research questions, all IC strategic plans influence the research directions and methods proposed by investigators in their applications. By the same token, strategic plans inform IC decisions about areas of research that require stimulation—achieved through a variety of means including meetings, workshops, conferences, Program Announcements, and Requests for Applications, and Requests for Proposals—to move science planning into the

implementation stage. Finally, strategic plans influence IC decisions on which applications to fund.

While each of the 24 grant-making ICs has a broad Strategic Plan that clearly states its mission and priorities, many of the ICs also have disease- and program-specific strategic plans and research agendas as well as reports from workshops, “blue ribbon” panels, and other expert panels that contain recommendations for research goals or priorities within the IC mission.

Strategic planning at NIH is a highly consultative process involving many constituencies that generate and provide input on public health needs and research gaps, opportunities, and priorities. Importantly, strategic plans also serve as a means for ICs to measure and report on portfolio balance and progress relative to their missions. NIH stays constantly tuned to the twin touchstones for priority-setting—public health need and state of the science. In Chapter 2, at the end of each disease topic section, are lists of relevant strategic plans.

Division for Planning and Strategic Initiatives

As noted above, the NIH Reform Act of 2006, signed into law in January 2007, created the new Division of Program Coordination, Planning, and Strategic Initiatives (DPCPSI) within the NIH/OD. The purpose of DPCPSI is to identify and report on research that represents important areas of emerging scientific opportunity, rising public health challenges, or knowledge gaps that deserve special emphasis and would benefit from the conduct or support of additional trans-NIH research (research that involves collaboration between two or more ICs), or would otherwise benefit from strategic coordination and planning. As specified in the NIH Reform Act of 2006, a Council of Councils, which met for the first time in November 2007, advises the NIH Director on matters related to the policies and activities of DPCPSI. To a large extent, the legislative mandate for DPCPSI confirms the administrative action that NIH took when it established OPASI. As such, OPASI will continue its role in developing and managing Roadmap initiatives as an integral part of DPCPSI.

Office of Portfolio Analysis and Strategic Initiatives

When NIH established OPASI in spring 2006, the aim went beyond institutionalizing the Roadmap planning and investment process. OPASI's full role is to provide NIH and the ICs with the methods and information necessary to manage their large and complex scientific portfolios; to lead trans-NIH efforts in identifying new and shifting public health challenges and important areas of emerging scientific opportunity; and to assist in accelerating trans-NIH investments in these areas, focusing on those involving multiple ICs.

OPASI develops and employs databases, analytic tools, and methodologies to conduct key assessments and portfolio analyses and integrates these analyses with information from multiple other sources for use in identifying and recommending concepts for trans-NIH initiatives. Since evaluation is an integral part of strategic planning and priority-setting, OPASI also is responsible for planning, coordinating, and conducting program and initiative evaluations. As part of its evaluation agenda, OPASI subjects each Roadmap initiative to rigorous review, with outcome tracking, an annual review of progress, and a major review not later than the fourth year of the initiative. In addition, OPASI is responsible for overseeing and coordinating IC use of evaluation set-aside funds and the systematic assessments required by the Government Performance and Results Act (Pub. L. No. 103-62) and application of the OMB Program Assessment Rating Tool.

Roadmap 1.5 and the Common Fund Strategic Initiative Process

Roadmap initiatives are a collective NIH-wide resource supported through the NIH Common Fund. They were previously funded through IC and OD contributions, but since FY 2007 have been funded within the OD appropriation level. While OPASI does not have direct grant-making authority, the Common Fund provides an “incubator space” for Roadmap and other initiatives on a time-limited basis (5 to 10 years). Initiatives either transition out to the ICs after this 5- or 10-year period or are concluded. In this way, NIH can remain nimble in

responding to newly identified emerging research needs that have the potential to transform biomedical or behavioral research.

To perpetuate the Roadmap, OPASI manages the [process by which recommendations for trans-NIH strategic initiatives are selected and developed](#), and provides the information needed for NIH leadership to allocate resources effectively for these trans-NIH efforts.

Criteria for Major Roadmap Initiatives

- Is the proposed initiative truly transforming — could it dramatically affect how biomedical and/or behavioral research is conducted over the next decade?
- Will the outcomes from the proposed initiatives synergistically promote and advance the individual missions of the ICs to benefit health?
- Does the proposed initiative require participation from NIH as a whole and/or does it address an area(s) of science that does not clearly fall within the mission of any one IC or OD program office?
- Is the proposed initiative something that no other entity is likely or able to do, and is there a public health benefit to having the results of the research in the public domain?

The process is exemplified by the steps OPASI took in 2006 and early 2007 to select and plan the next generation of Roadmap initiatives—Roadmap 1.5—which will be funded in FY 2008.

Through summer and fall 2006, NIH solicited ideas for new initiatives from the intramural and extramural scientific community, patient advocates, and the general public to help senior NIH staff identify crosscutting challenges in biomedical research that meet special criteria established for Common Fund (Roadmap) initiatives. One of the important steps in this process was issuance of a Request for Information published in the NIH Guide ⁸ in October 2006. The respondents were invited to submit up to three ideas that met predetermined criteria to be considered for a Roadmap initiative.

To facilitate the prioritization of ideas, OPASI coordinated a programmatic review of the submitted ideas assessing their responsiveness to the criteria. To further inform the decision-making process, OPASI and ICs worked together to provide a preliminary assessment of the currently funded portfolio of research related to several of the broad areas highlighted by the ideas. OPASI efforts to develop portfolio analysis tools will enhance NIH capacity for these analyses. Informed by this analysis and following extensive scientific discussion, the IC Directors selected broad areas that were to be pursued either as major Roadmap initiatives, pilot studies, coordination areas, or strategic planning areas.

Next, Trans-NIH Working Groups, led by IC Directors, developed specific proposals in the identified broad areas. Then, in May, the IC Directors and NIH Director met to review and prioritize the proposals. They selected two topics, the Microbiome and Epigenetics, for immediate implementation as 5-year Major Roadmap Initiatives. Although the Reform Act was not enacted in time for NIH to establish and convene the Council of Councils during consideration of concepts for Roadmap 1.5, in the future the Council will act as an external advisory panel to the IC Directors during the concept approval stage of Roadmap initiatives.

- **Microbiome.** The Microbiome is the full collection of microbes (e.g., bacteria, fungi, viruses) that naturally exist within and on the human body. In a healthy adult, microbial cells are estimated to outnumber human cells by a factor of 10 to 1. These communities, however, remain largely unstudied, leaving almost entirely unknown their influence on human development, physiology, immunity, and nutrition. The NIH Human Microbiome Project (HMP) will generate the tools and resources necessary for comprehensive characterization of the human microbiota and analysis of their relationship to human health and disease.

- **Epigenetics/Epigenomics.** Epigenetics is the study of changes in gene expression or function that are caused by factors other than change in DNA sequence. Epigenetic changes allow cells to have different characteristics despite containing the same genomic material. Curiously, some epigenetic features are inherited from one generation to the next. This subject is of such current scientific interest that the prominent journal *Cell* recently devoted an entire special review issue to the subject⁹. The overall hypothesis of the NIH Epigenomics Program is that the origins of health and susceptibility to disease are, in part, the result of epigenetic regulation of the genetic blueprint. Initiatives in this area would develop a “toolbox” to better measure these genetic modifications; collect data and develop algorithms to build the infrastructure needed to model epigenetic processes; and incorporate epigenetic information in genetic studies to increase our understanding of the relationship to human health and disease.

Other topics were approved as pilot studies, coordination areas, and strategic planning areas (see Appendix C, the *Common Fund Strategic Planning Report, FY 2008*, for further information on plans for use of the Common Fund).

⁵For more information, see <http://officeofbudget.od.nih.gov/PDF/Significant%20Items-2008.pdf>

⁶For more information, see <http://www.healthypeople.gov/>

⁷For more information, see http://www.hhs.gov/strategic_plan/

⁸For more information, see <http://grants.nih.gov/grants/guide/notice-files/NOT-OD-07-011.html>

⁹*Cell*, 128, February 23, 2007.