



**Testimony
Before the
Committee on Energy and Commerce
United States House of Representatives**

**LEGISLATIVE
REAUTHORIZATION OF THE
NATIONAL INSTITUTES OF
HEALTH**

Statement of

Elias A. Zerhouni, M.D.

Director

National Institutes of Health

U.S. Department of Health and Human Services



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Chairman Barton and Members of the Committee. I am Dr. Elias A. Zerhouni, Director of the National Institutes of Health (NIH), and I am here today at your request to testify about the legislative reauthorization of NIH. The current reauthorization proposal would be only the third omnibus reauthorization of NIH since enactment of the Public Health Service Act in 1944. Omnibus reauthorizations occurred in 1985 and 1993. Those previous acts expanded the number of Institutes and Centers at NIH, concentrating on specific diseases, organ systems, and special populations.

As a result of such structural growth and appropriation increases, highlighted by the doubling of NIH's budget between 1998 and 2003, the NIH is a far different organization than it was 13 years ago, when Congress last reauthorized Title IV of the Public Health Service Act. Our budget is nearly \$29 billion. We have over 17,000 employees.

Throughout its history, NIH drove the biomedical research engine of our Nation toward unprecedented scientific discoveries that improved public health and fundamentally changed the nature of medicine as well as the burden of disease. Our success in addressing acute illnesses has shifted the landscape of disease from once acute, severe, and lethal conditions to more chronic and manageable conditions.

However, as the Institute of Medicine observed in 2002, "While NIH's success is to be celebrated, success alone does not answer fully the question of whether there is a better way to proceed, particularly as one faces a future where the world of biomedical sciences is being rapidly transformed in all its dimensions."

The key transformation has been the convergence of scientific concepts, approaches, opportunities, and needs across all diseases and conditions. As we have learned more about the molecular causes of diseases, we have found great similarities between the mechanisms that lead to diseases once thought unrelated. Often, research in one field finds unexpected application in another. The greatest research advances of recent years involve the fields of molecular and cell biology as well as genomics and proteomics, among others. Their applications will not be limited to specific diseases or populations. They will be applied to all diseases and all populations. This will require greater interdisciplinary efforts. NIH strives to encourage these new ways of conceptualizing and addressing scientific questions and to encourage their translation from the laboratory to the clinic. At the same time, we work towards increasing our understanding of the behavioral and social sciences necessary to insure the success of biological approaches to health and disease.

For example, the convergence of science underlies the new Genes and Environment Initiative in NIH's FY 2007 budget request to Congress. This is a project designed to address a broad array of health and disease concerns and will build on advances in multiple areas of science, including genomic sequencing technology and environmental science. It will give us the unprecedented ability to discover the potential causes of the 10 most common diseases afflicting the U.S. population. Already such approaches are yielding a trove of discoveries in areas from mental disorders, to cancer, to the prevention of age-related blindness.

We have great expectations for the advancement of biomedical research in the coming years. The question now being asked by Congress, the scientific community, medical providers, patients, and NIH itself is: does the current structure of NIH allow the multi-disciplinary and collaborative approach to science required to meet these expectations? In this era of enormous potential and scientific convergence, how does NIH best adapt?

Fundamental science has rapidly evolved due to recent advances in new fields such as genomics, proteomics, and many other breakthrough discoveries. The boundaries between the specific science areas of each of NIH's 27 Institutes and Centers (ICs) are increasingly blurred and now require greater interdisciplinary interactions. Our population faces chronic and complex diseases, which now account for over 75% of healthcare expenditures. Patients often suffer from more than one disease at a time affecting multiple organ systems, mechanisms, and life stages, creating the need for greater coordination. In many ways, ICs have already responded and are working together whenever appropriate.

Better mechanisms of functional integration that enhance synergy across all of NIH need to be found. Some provisions in the proposed bill serve this purpose by creating a common and shared mechanism for addressing issues that no single IC can address, and providing opportunities for ongoing formal review of the structure of NIH through input from IC Directors, scientific advisors, and other stakeholders. Over the past 4 years, NIH has experimented with ways to accomplish these goals by implementing a series of trans-NIH initiatives such as the Roadmap for Medical Research, the NIH Plan for Obesity Research, the Neuroscience Blueprint, and many other initiatives, all designed to take rapid advantage of the enormous progress made during the doubling of the NIH budget. Establishing these formal mechanisms of integration gives NIH a great opportunity to build on its remarkable success to date.

The bill under consideration fosters interdisciplinary research and strategic planning by establishing an organization to integrate the work of the ICs through the identification of trans-NIH research programs that will broadly impact all areas of research. Further, the bill would create a funding mechanism – a common fund for shared purposes – for greater coordination of NIH research, whenever appropriate, as determined through an open and collaborative consultation and advisory process involving all relevant stakeholders. This fund is in conformity with the Common Fund for shared needs that NIH has already established to support trans-NIH initiatives, as discussed below. The bill preserves the time-honored NIH system of peer review and evaluation.

I believe the current proposal will preserve such vital authorities as peer review and the pursuit of scientific opportunity through investigator-initiated grants - which have been and should remain the mainstay of our research support mechanisms. I believe strongly that free exploration of ideas generated by the scientists themselves is the key to our long term success. Human subjects protections, and the requirement to disseminate research findings to the public will remain, as in our current authorization. I think the Committee is focused on organizational efficiency and effectiveness, which is the principal challenge for an increasingly large and complex organization.

This bill creates a central planning and analysis division for trans-NIH research within the Office of the NIH Director. NIH has recently established such an office through administrative mechanisms. It will be instructive to the Committee to share NIH's vision for the function of this new organization.

The mission of the new Office for Portfolio and Analysis and Strategic Initiatives (Office) is to provide NIH and its constituent ICs with the methods and information necessary to manage their large and complex scientific portfolios, to identify – in concert with multiple other inputs – important areas of emerging scientific opportunities or rising public health challenges and to assist in the acceleration of investments in these areas. Bringing together these diverse components of the agency will facilitate “functional integration” of NIH in a time of unprecedented scientific opportunities. It will help the agency to increase its effectiveness and efficiency in advancing science, ultimately resulting in the acceleration of basic research discoveries and speeding the translation of those discoveries into applications that improve the health of the American people.

The Office will accomplish its mission through the activities of three divisions and an office of the director. The mission of the Division of Resource Development and Analysis (DRDA) is to employ resources (databases, analytic tools, and methodologies), and to develop specifications for new resources, when needed, in order to conduct assessments based on NIH and other databases in support of portfolio analyses and priority setting in scientific areas of interest across NIH. DRDA will also be a resource for portfolio management at the programmatic level, should individual ICs request the Division's expertise or tools.

The Division of Strategic Coordination (DSC) is responsible for integrating information and managing the process by which recommendations are developed to inform the priority-setting and decision-making processes of the NIH in formulating trans-NIH strategic initiatives. These initiatives will address exceptional scientific opportunities and emerging public health needs. The DSC will provide the Director with the information needed to allocate resources effectively for trans-NIH efforts. Although the new office will not have grant-making authority, the DSC will provide an “incubator space” for trans-NIH initiatives, and support priority projects on a time-limited basis (generally 5 years and not to exceed 10 years). This will support continuous development of new, trans-NIH efforts adaptive to public health and scientific opportunities and issues through all available mechanisms, including individual investigator-initiated research grants as determined by scientific consultations. Support will come from pooled resources (the Common Fund).

The Division of Evaluation and Systematic Assessments (DESA) will plan, conduct, coordinate, and support program evaluations, including, but not limited to, Institute and Center-specific program and project evaluations; evaluations of trans-NIH activities, including Roadmap initiatives; and systematic assessments, such as those required by the Government Performance and Results Act (GPRA) and the OMB Program Assessment Rating Tool (PART). The functions of DESA will allow for strategic planning and the coordination and evaluation of the NIH research agenda and portfolio and provide essential information for determining NIH-wide resource allocations.

The Office will make use of a “Common Fund” for shared NIH needs. The Common Fund is an annual set-aside fund created from an agreed-upon percentage of the annual budgets of each of the NIH ICs to support activities/efforts identified by the Office. Office operations will not be funded out of the Common Fund.

Stakeholders, including the scientific and advocacy communities, will be invited to submit ideas for new initiatives on a regular basis. These nominations will be considered by the NIH leadership, external consultants, IC Directors, representatives of IC advisory councils, and other advisory councils. Once a new initiative is approved, it will be assigned to a lead IC for further development and administrative oversight. Funds from the Common Fund will be used to support the initiative. The progress of each initiative will be subject to rigorous review. There will be an annual review of progress and a major review at year 3-4 that will determine, not later than year 5, whether to renew the initiative for a final 5-year period, continue the research but transfer support to a more appropriate Institute or Center, or complete the initiative. No initiative will remain for more than 10 years, thus insuring the long-term flexibility and vitality of this approach.

I remind the Committee that Title III of the Public Health Service Act authorizes the Secretary of Health and Human Services to use the Public Health Service to “encourage, cooperate with, and render assistance to other appropriate public authorities, scientific institutions, and scientists in the conduct of, and promote the coordination of, research, investigations, experiments, demonstrations, and studies relating to the causes, diagnosis, treatment, control, and prevention of physical and mental diseases and impairments of man”

Over the past 50 years, the achievements of NIH and our academic and industry partners in medical research are nothing short of remarkable. According to the latest report on the Nation’s health from the Centers for Disease Control and Prevention (CDC), life expectancy continues to rise, now at an unprecedented 78 years for the total U.S. population. Since 1950, the age-adjusted death rate for the total population declined by a remarkable 43 percent. Life expectancy has increased by one year in every five for the past 30 years. Americans are not only living longer, they are healthier. For instance, the disability rate of American seniors dropped by almost 30 percent in the past 20 years, owing to a range of scientific advances.

In the past 30 years, death rates of two leading killers, cardiovascular disease and stroke, have declined by 63 percent and 70 percent, respectively. Such medical breakthroughs as drug-coated stents, therapies to achieve safe levels of blood pressure, and cholesterol lowering drugs have cut the expected number of deaths from heart attacks this year by more than half. In the past year alone, more than a million lives were saved.

For patients affected with AIDS, the development of highly active antiretroviral therapy (HAART), the result of work performed by a cadre of NIH-supported scientists and their counterparts in industry, has transformed AIDS into a manageable disease, preventing hundreds of thousands of hospitalizations and early deaths. The advances have had a particular impact on children. Today, fewer than 50 HIV-infected babies are born each year in the United States, sparing 16,000 to 20,000 infants from mother-to-child AIDS transmission.

This year, for the first time in history, the absolute number of cancer deaths in the U.S. has decreased. We now have ten million cancer survivors. We can detect and treat cancer at earlier stages. Targeted therapies have emerged, using specific molecular targeting to treat tumors with new agents. NIH's National Cancer Institute and others have identified biomarkers of cancer, foreshadowing an era when the disease can be predicted before symptoms appear, and treatment can be effectively targeted and personalized to the individual cancer patient.

For the first time in history, scientific progress allows us to hope for a revolutionary era when medicine will move from being **curative** and inherently costly in nature to become ***predictive, personalized, and preemptive***. Toward this goal, NIH is strategically investing in research to further our understanding of the fundamental causes of diseases at their earliest molecular stages so that we can reliably ***predict*** how and when a disease will develop and in whom. Because we now know that individuals respond differently to environmental changes according to their genetic endowment and their own behavioral responses, we can envision the ability to precisely target treatment on a ***personalized*** basis. Ultimately, this individualized approach, completely different than how we treat patients today, will allow us to ***preempt*** disease before it occurs with the hope of reducing future healthcare costs. Our vision is simply to transform medicine and health through accelerated discoveries.

This concludes my testimony. I will be pleased to respond to any questions Members of the Committee have.