

DEPARTMENT OF HEALTH AND HUMAN SERVICES

NATIONAL INSTITUTES OF HEALTH

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Good morning, Mr. Chairman and members of the Committee. Let me begin by thanking the Committee for your continuing support of the National Institutes of Health (NIH). Through your unprecedented doubling of our budget, we have been able to support remarkable, life-saving research. I will share some of our recent breakthroughs with you and discuss our strategic approach to dealing with the dual challenges of deficit reduction and increasing health concerns. This past year at NIH has been a time of extraordinary collaboration, progress, and discovery that will help transform medicine in the 21st century.

NIH AND 21ST CENTURY MEDICINE

Let me describe the many challenges the future will bring to medical research. Our population is aging rapidly. Chronic diseases now account for 70% of all deaths and 75% of our health care costs. Our discovery of new, affordable treatments can extend our productive years and reduce the burden of disease. This depends on preventive strikes against disease, on the intervention of science *before* symptoms appear and *before* normal function is lost. We *are* making progress, and I want to walk you through some highlights.

RECENT NIH PROGRESS AGAINST DISEASE CHALLENGES

For the first time, due to remarkable progress, heart disease has dropped below cancer as the leading cause of death for Americans under age 85. This is in spite of the advances we have made in combating cancer. Cancer survival rates are improving while incidence and mortality decline. Five-year survival rates have substantially

improved in the past two decades, and cancer death rates have dropped 1.1% per year between 1993 and 2001. Since the early 1990's, death rates have decreased for 11 of the top 15 cancers in men, and eight of the top 15 in women. Lung cancer deaths rates among women leveled off for the first time between 1995 and 2001, after continuously increasing for many decades.

A new test can predict both the risk of breast cancer recurrence and may identify women who will benefit most from chemotherapy. Results suggest that almost half of the more than 50,000 U.S. women diagnosed every year with estrogen-dependent, lymph-node negative breast cancer may not need to go through the discomfort and side effects of chemotherapy because their risk of recurrence is so low. This is important news for many women and their families.

In a "proof-of-principle" study, scientists found they could measure elevated levels of four distinct cancer-associated molecules in saliva and distinguish with 91 percent accuracy between healthy people and those diagnosed with oral squamous cell carcinoma. This marks the first report in the scientific literature that distinct patterns of the body's primary macromolecules, known as messenger RNA, are not only measurable in saliva--but can indicate a developing tumor.

A new imaging system is under development that may shorten the length of surgical procedures and allow surgeons to more precisely pinpoint, and then remove, damaged brain tissue that causes seizures. A multi-institutional team of engineers, scientists, and physicians, may improve the chances of successful surgery for some epilepsy patients. The same kinds of techniques are bringing new hope to patients with Parkinson's disease through deep brain stimulation.

We are continuing to make investments in understanding and treating chronic diseases such as diabetes, Alzheimer's disease, Parkinson's disease, and hypertension. A long-term study has found that diabetes mellitus was linked to a 65% increased risk of developing Alzheimer's disease (AD). Current research underway will tell us whether therapies for diabetes may play a role in lowering the risk of AD or cognitive decline. We have also developed a comprehensive plan to understand, treat and hopefully prevent autism, a daunting problem.

The research that we are conducting in molecular biology and molecular genetics to identify disease early and intervene before irrevocable damage occurs will provide similar payoffs in this new century. We hope to be increasingly able to thwart diseases as they are emerging years before they strike, at potentially greatly reduced costs.

As we find new molecular signposts for risk and early signs of disease, we can lower the burden of disease in a more cost-efficient way. Our ability to predict and intervene will be orders of magnitude more effective and can ultimately reduce long term health care costs. But moving to this new model of medical research will require vision, management, and scientific progress.

SCOPE OF THE CHALLENGE

The American taxpayer generously invests \$96 per person, per year in the NIH for the future health of our country. To give a sense of perspective about our task, I look at the total \$29 billion for NIH – nearly half the discretionary funding for all of HHS—and I know I need to make sure that such a large investment is used in a

strategic and systematic way to maximize the chances that better treatments and cures are rapidly developed to stem the rising burden of disease, as health care costs consume over \$5,500 per person per year and are rising. NIH is currently using this investment for research to attack hundreds of common diseases and an estimated 6,000 rare diseases or conditions that affect approximately 25 million people in the United States - - diseases and disorders that may strike any of us, our parents, our children or our friends. In recent years, we have taken on new challenges such as biodefense.

NIH has strengthened its vision, tightened its management, and unified its scientific efforts. Over the past three years, I have presented to you examples of major shifts toward a renewed vision that spans the NIH Institutes and Centers: *The NIH Roadmap* in FY2004, the *NIH Strategic Plan for Obesity Research* in FY 2005, and now, the *NIH Neuroscience Blueprint*.

STRENGTHENING THE NIH VISION

NIH Roadmap for Medical Research. Two years ago, I announced the *NIH Roadmap for Medical Research*. The Roadmap's overarching goal is to accelerate research and translate the scientific results that are ready for implementation into cures. It is a trans-NIH effort to drive progress in medical research while transforming the way science is conducted. Through the Roadmap, we are supporting research initiatives that no single institute could tackle alone, but that will tangibly benefit all of NIH.

For example, to speed the translation of scientific discoveries into health care interventions, Roadmap efforts are underway to improve and extend clinical research network capabilities and embrace a growing "community of research." Roadmap

initiatives will make more powerful use of data. We will illuminate approaches to standardized clinical research data reporting and data-and-sample sharing across diseases and health care settings. We will elicit “best practices” from existing clinical research data networks and test their interoperability. For example, The Patient Reported Outcomes Measurement Information System (PROMIS) will enhance how we measure and manage health improvements as reported by patients themselves by standardizing the way we track symptoms that transcend many chronic diseases and conditions. A network of biocomputing centers is integrating informatics, physical and biologic technologies for ready access by researchers to harness the increasing quantity and complexity of biomedical and behavioral scientific data.

New tools such as biochemical and imaging probes will illuminate the complex series of events that lead to disease, providing a host of new therapeutic targets. Through the Roadmap, there also will be an infusion of trained scientists and clinical researchers, able to apply interdisciplinary and multidisciplinary approaches to complex biomedical problems.

NIH Strategic Plan for Obesity. NIH has taken on one of the nation’s most dramatic health challenges---obesity. The most recent figures show that 65 percent of U.S. adults — or 130 million people — are either overweight or obese. In addition to decreasing quality of life and increasing the risk of premature death, obesity and overweight will cost the Nation an estimated \$117 billion in direct medical costs and indirect costs such as lost wages.

Obesity is not an easy problem. It requires multiple approaches. The obesity epidemic represents a complex interplay of behavioral, sociocultural, economic, and

environmental factors against a backdrop of genetic and other biological factors. The *NIH Strategic Plan for Obesity Research* exploits the breadth of expertise available from a wide range of disciplines at the NIH, engaging 19 Institutes and Centers to set an agenda for this research.

NIH-supported scientists are continuing to make significant progress in obesity research. For example, our investigators are identifying an elaborate network of hormones and other molecules that connect the brain, gastrointestinal tract, fat cells, and other parts of the body to achieve energy balance. An increased level of one of the appetite-inducing hormones was found in obese people following diet-induced weight loss. It may explain why some people may have difficulty “keeping weight off” after dieting. Such hormones are now “targets” for drug design. New drugs could affect appetite, food absorption, and/or energy expenditure.

NIH Neuroscience Blueprint. Major causes of human suffering and the burden of disease include mental illness, neurological disorders, and a range of behavioral disorders. Together, these illnesses exact a cost of more than \$500 billion a year. Focusing on how NIH does neuroscience research, the Directors of 15 Institutes came together and drafted the *NIH Neuroscience Blueprint*. This model of strategic leadership and cooperation addresses several of the most common causes of death and disability, as well as hundreds of rare disorders that affect the brain, spinal cord, or nerve cells throughout the body, including drug abuse, alcoholism, Alzheimer’s disease, Parkinson’s disease, ALS or Lou Gehrig’s Disease, chronic pain conditions, developmental disorders, dementias of aging, and a myriad of problems of hearing, vision and other senses. The Blueprint capitalizes on economies of scale to create

resources, tackle common scientific problems, and train the next generation of neuroscientists. *The NIH Roadmap*, the *NIH Strategic Plan for Obesity Research*, and the *NIH Neuroscience Blueprint* represent three milestones in a new strategic use of resources.

NIH RESPONDS TO EMERGING HEALTH THREATS

New infections are spreading to new places and new populations across the world, and NIH research is responding to breaking medical challenges on all these fronts. In addition, we are living with bioterrorism threats. During FY 2006, NIH is developing countermeasures to address microbial, nuclear, radiological, and chemical threats. At the same time that scientists are investigating these emerging challenges, they are learning about mechanisms of a wide range of disease processes.

Recently, NIH-supported investigators uncovered the three-dimensional structure of the anthrax toxin complex, going from gene to structure in an astonishing three weeks and giving scientists a potential target for blocking the toxin's deadly effects. In addition to anthrax, we are deeply concerned about the other "Category A" microbes -- smallpox, plague, tularemia, hemorrhagic fevers, and botulinum toxin -- that can be deadly and can be deployed as bioweapons.

Because of the dramatic ways that disease-causing viruses, bacteria, and parasites continue to change over time--positioning the NIH for rapid response is also critical. Alarmed, we see new pathogens emerge--and familiar ones re-emerge. AIDS, West Nile virus, monkeypox, avian influenza, and SARS, are examples. In this past month, newspapers have been filled with reports about avian flu. NIH is vigorously

supporting research to develop responses to these threats. Last year I described how we responded, within weeks, to SARS. I am pleased to report that one of the three new NIH-developed candidate vaccines is now in its first human clinical trial.

Last year, worldwide, 4.9 million people became infected with HIV and 3.1 million died due to AIDS. We have developed treatments that transformed HIV/AIDS from a death sentence into a debilitating chronic disease. We must do better—we must prevent HIV infection. We have been working for years to develop an HIV/AIDS vaccine. Now, we have six viable candidates and testing them is our highest priority. Accordingly, we plan to increase the portion of our budget devoted to HIV/AIDS vaccines by \$100 million or 20% to seize this ripe scientific opportunity for progress.

I have described several ways that the NIH vision has been transformed. It has been moved by discovery, shifted by disease burden, and has been transformed to adapt to both threat and need. A former colleague of mine has contrasted the roles of physicians who save lives “one patient at a time” with those who serve in public health and who save “millions of lives at a time.” At NIH, and through our investigators in every state and through their more than 3,000 institutions that include universities, medical schools, hospitals and other research facilities, we are providing the research firepower to do *both*. We depend upon the creativity and rigor of more than 200,000 scientific investigators, supported by the NIH appropriation you continue to provide.

MANAGING FOR THE 21ST CENTURY

As the 21st century begins, we have re-tooled our management tactics. I have just met with the 27 IC directors to revisit our operations and to adjust our policies to

make full use of the combined skills of our IC leaders. We are, simultaneously, streamlining our decision-making processes across the enterprise.

PART. NIH is understandably proud that major components of our largest activity, the Extramural Research Program, which supports research all across the country and accounts for \$24 billion of our appropriation, received the highest possible rating of “Effective” in the FY 2006 Program Assessment Rating Tool (PART).

Investigator-Initiated Research. Individual research grants remain the mainstay of NIH investment, and research in priority areas will always be awarded competitively. We are also committed to supporting new investigators, who without support, might be lost to the continuum of discovery.

Portfolio Analysis. The agency is successfully engaging in trans-NIH initiatives such as the Roadmap, Obesity, and the Neuroscience Blueprint. It is, however, time to focus additional attention on creating better institutional tools to analyze, assess, and manage the NIH-wide research portfolio and to provide better information to support priority-setting decisions in areas of common interest to all Institutes and Centers. We are creating an Office of Portfolio Analysis and Strategic Initiatives to focus such efforts, which will include an improved process for collecting data on various diseases, conditions, and research fields, and improvements in data about burden of disease. The staff will seek broad public input—from the public, health care providers, policymakers, and scientists—in addition to soliciting advice from within the NIH and making more coordinated and effective use of the NIH-wide evaluation process.

More effective analysis and management of our portfolio will lead to even better progress against disease. *Ninety-six dollars per person per year.* This is our challenge--to make the taxpayer's generous contribution to NIH, \$96 per person per year, stretch as far as is feasible and to leverage as much discovery as is possible since each one of us some day may fall prey to disease. We have also responded to challenges and opportunities that are integral to public trust.

RESPONDING TO PUBLIC TRUST CHALLENGES AND OPPORTUNITIES

Conflict of Interest. NIH has addressed conflict of interest challenges with sweeping reform. Once we began a systematic investigation of what had initially appeared to be some isolated cases, we realized that conflict of interest was a serious problem that needed management and reform. Strict and clear rules needed to be put in place. Nothing is more important to me than preserving the public's trust in NIH. I am confident that the new HHS rules that were put in place last month will prevent the recurrence of past abuses and will go a long way toward preserving the historic role of NIH as the primary source of unbiased scientific health information for the country. We must have the public's trust, and we must continue to earn it. In this, we will carefully evaluate the impact of these new rules on our ability to recruit and retain the best scientists and adjust our policies accordingly while maintaining first and foremost our commitment to preserve the role of NIH as an unimpeachable source of scientific advice.

Public Access. On February 3, NIH announced our historic public access policy. For the first time, the public will have access to peer-reviewed research

publications that result from studies funded by NIH, and scientists will have a new pathway to make their NIH funded research known to the public that funded it. Starting in May of this year, research results will be more readily accessible to you, your constituents, your staff, the public at large, health care providers, educators, and scientists. We are moving to maximize participation while encouraging scientists to submit their final manuscripts as soon as possible, within 12 months of final publication. The policy creates a permanent archive to preserve vital research findings, which will be a searchable compendium of these research publications. The compendium will be used to manage NIH more efficiently and to help our scientists and many other stakeholders better understand the NIH research portfolios, monitor scientific productivity, and, ultimately, help set research priorities.

BUDGET

The discretionary FY 2006 request for the NIH appropriation is \$28,590 million, an increase of 0.5% over FY 2005. In addition, a \$50 million increase is included in the request for the Public Health and Social Services Emergency Fund (PHSSEF) for NIH research in chemical countermeasures, bringing the total increase for NIH to \$196 million, or 0.7%. The request sustains funding for important initiatives put forth in recent years while addressing the need for spending discipline to support the Administration's initiative to cut the deficit in half over four years. This request also represents our first integrated performance budget.

The NIH Budget for FY 2006 has five high priorities. Research project grants increase by \$56 million, raising the number of new and competing research project

grants by 247 grants to a total of 9,463 in our effort to preserve to the greatest extent possible the ability of scientists to obtain individual support, a critical goal. The Roadmap for Medical Research adds \$98 million to total \$333 million to keep this multi-year initiative on track, so we can accelerate research toward treatments and cures -- the majority of this funding will also go to individual as well as teams of researchers. To develop countermeasures against biological and chemical threats, biodefense research increases by a net \$56 million to \$1.8 billion. The Neuroscience Blueprint that supports NIH Institutes collaborating to use new tools to solve some pressing public health challenges receives \$26 million. The highest priority in combating HIV/AIDS is the development of a vaccine, for which funding rises by \$100 million to \$607 million.

TRANSFORMING MEDICAL RESEARCH

We are proud of our progress, but we know how much is left to be done. Health disparities remain a challenging problem for our country. Part of the reality is that some things never happen as quickly as we might hope. We continue to manage for change in rapidly changing times and amidst unprecedented scientific opportunities. We are focused on shared resources, clear objectives, and the stronger teams needed to tackle the daunting complexity of biology. We are creating a strategic framework for future discovery. As the nation's medical research agency I am confident that we will continue our record of improving health through discovery at an accelerated pace in the 21st century.

Thank you for this opportunity to testify. I would be pleased to answer any questions.