

**DEPARTMENT OF HEALTH AND HUMAN SERVICES
NATIONAL INSTITUTES OF HEALTH**

Fiscal Year 2003 Budget Request

**Witnesses appearing before the
House Appropriations Subcommittee on Labor-HHS-Education**

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I. INTRODUCTION

Mr. Chairman and Members of the Subcommittee: I am Dr. Andrew von Eschenbach, the Director of the National Cancer Institute (NCI). I am pleased to appear before you to discuss some of the activities supported by the NCI and to present the President's budget proposal for fiscal year 2003. The significant budget increases over the past several years have allowed the NCI to continue on an aggressive path of discovery in cancer research. This path is aimed at the development of interventions that will continue to reduce the suffering and death caused by cancer.

Over the past 30 years, our nation has invested a great deal of its resources in cancer research. It is an investment that has enabled the NCI to conduct research and to support thousands of scientists throughout this country. It is an investment that has sustained promising research and more recently, data-sharing infrastructures and multidisciplinary collaborations. And it is an investment that is now paying significant scientific dividends. Where major breakthroughs were once measured in years or even decades, we are now moving forward at record pace. Every day, we uncover yet another footprint in the genetic and molecular process by which a cell becomes malignant, grows uncontrolled, invades, metastasizes, and ultimately kills.

While our knowledge of this complex process is still rudimentary, the path ahead is now clear and greater dividends are within reach. Even with our just emerging picture of cancer, we are exploiting this knowledge to devise better imaging and diagnostic tools and design new interventions to treat and prevent this devastating disease.

We stand on the threshold of a biomedical revolution, where multidisciplinary collaboration will translate the breakthroughs of basic research swiftly from the lab to the bedside. One recent example of success emerged in the fight against ovarian cancer, one of the deadliest cancers for women, in part due to lack of effective screening methods. A

sophisticated computer-based screening tool has shown the ability to recognize protein profiles in the blood from women with diagnosed ovarian cancer and uses the information to detect new cancer cases in women at an early stage of disease. Current discovery of such molecular signatures of cancer may also make possible powerful, new tools for detecting cancer and its recurrence.

The elucidation of the biology of cancer is a scientific pursuit. But the eradication of cancer is a human experience. The ultimate goal of the people of the National Cancer Institute is saving lives and improving the quality of life among cancer patients.

II. CANCER TRENDS

Five years ago, NCI initiated an annual report to the Nation on the burden of cancer. This report is developed in collaboration with the American Cancer Society (ACS), the North American Association of Central Cancer Registries, the Centers for Disease Control and Prevention and its National Center for Health Statistics. Based on statistics from these sources, we are continuing to see encouraging overall trends, including continued decline in the rate of new cancer cases and cancer deaths.

Today, we can successfully treat or increase life expectancy for more than half of all cancer patients. We now have more options for prevention, including chemoprevention such as tamoxifen for breast cancer, and are developing more evidence-based interventions for cancer control. Adult smoking is down dramatically from the 1960s for men and the increase in smoking among women has finally reached a plateau. The latest statistics from the Report to the Nation that we will release this spring also show that while breast cancer incidence continues to rise (due to increase in early stage disease), overall breast cancer deaths continue to decline. And for the first time ever, we are seeing a small, but significant decline in breast cancer mortality among African-American women.

Yet even as these trends give rise to hope, they must also steel our resolve to use the fruits of discovery to the further benefit of patients. That's because we know that this year, based on ACS estimates, over 1.2 million Americans will be diagnosed with cancer this year, and about 550,000 Americans are expected to die from this disease, more than 1,500 people a day. The number of new cancer cases is still rising for some cancers such as esophageal, liver, melanoma, and non-Hodgkin's lymphoma. And there remains a disparate burden of cancer experienced by America's underserved population. Another trend indicates that youth smoking continues to rise except in states with vigorous tobacco control programs. NIH estimated the overall costs for cancer to be \$156.7 billion in the year 2001.

Of course, behind these numbers lies the real and human face of cancer. It is the face of a child with retinoblastoma whose only hope is radical surgery that will leave him cured but permanently blind. It is the face of a young woman living with the fear that her breast cancer will recur. And it is the face of a grandfather whose lung cancer has shattered his dream of spending his golden years with his grandchildren.

These faces demand urgency. It is an urgency that will be at the forefront of NCI's continued efforts to translate research quickly and safely to the cancer patient. I have highlighted several activities that illustrate NCI's accelerated approach to scientific discovery.

III. HIGHLIGHTS IN CANCER RESEARCH

We understand that improved technology for early detection and diagnosis is critically needed for cancer to become a rare disease. For this reason, imaging research supported by NCI is advancing on several fronts. Now, with the recent reawakening of debate on mammography guidelines, it is more important than ever to redouble our efforts in this area. In addition to assuring women that the weight of the evidence still shows that mammography saves lives, NCI is accelerating research into better screening tools. Besides efforts to improve conventional and digital X-ray mammography, NCI supports research for several other technologies such as magnetic resonance imaging (MRI), ultrasonography, positron emission tomography (PET), and single photon emission computed tomography (SPECT). Already, with these technologies, scientists can "see" biological processes taking place in living tissues such as blood flow, oxygen consumption, and glucose metabolism.

A major research effort is also under way to create molecular imaging technologies that can noninvasively detect and display the actual molecular events taking place in the body. Imaging technology to detect cancer recurrence using flurodeoxyglucose (FDG) PET scans and dynamic MRI for functional therapy monitoring are among the sophisticated imaging techniques currently being investigated.

In addition, several PET studies are in progress for the evaluation, staging and monitoring of therapy using PET for woman with breast cancer. In a large clinical trial from the University of Pennsylvania, doctors are incorporating dedicated breast PET into the standard diagnostic regimen for women with breast cancer.

On the therapeutic front, researchers are making headway against certain forms of leukemia, where an abnormal protein complex called bcr-abl forms inside the cell and stimulates uncontrolled growth. A search for agents that would interfere with bcr-abl led to the identification of STI-571, later renamed imatinib mesylate (Gleevec®). In clinical trials with this drug, more than 50 percent of patients with myeloid blast crisis responded well as measured by a decrease in the abnormal leukemic blood cells. Gleevec® has moved swiftly from clinical trials to the cancer centers and is now available as treatment for patients with chronic myelogenous leukemia (CML). This drug is now being evaluated in the treatment of ovarian, certain types of brain cancer, as well as a very rare form of stomach cancer and prostate cancer.

In the area of prevention, research is pointing to certain agents that are capable to changing a person's risk for cancer. When basic research establishes a biological basis for an intervention, trials serve to test the hypothesis. For example, the Selenium and Vitamin E Cancer Prevention Trial (SELECT) will determine if seven or more years of

daily supplements of selenium and/or vitamin E reduces the number of new prostate cancers diagnosed in healthy men. In addition, a Study of Tamoxifen and Raloxifene (STAR) will determine whether the osteoporosis drug raloxifene has equivalent breast cancer risk reduction benefits with reduced risk of side effects as compared to tamoxifen.

While the fast pace of discovery from these and other areas is welcome, the volume of data generated can often be overwhelming to the research community. To address this, NCI supports a fully integrated cancer biology approach to discovery through a discipline called bioinformatics. NCI programs such as the Cancer Genome Anatomy Project (CGAP), the Proteomics Initiative, Mouse Models Program, the Drug Discovery Program produce information and enable the research community nationwide to access these Web-based data sets that serve as tools for collaboration and scholarly discovery. This ensures that the analyses and interpretation of data across disciplines proceed in parallel and synergistically so that discovery in one system informs research in the other.

Bioinformatics enables researchers in CGAP to build, analyze, and interpret databases of genes expressed in cancer cells and of single nucleotide polymorphisms (SNPs), important markers for cancer risk-related genes. In proteomics, the ovarian detection tool that I mentioned earlier has demonstrated the power of bioinformatics to detect invisible patterns of disease. And in drug discovery, bioinformatics ensures that the most promising targets identified in the extramural research community can be exploited using the modern tools of cell-based drug analysis and gene-based high-throughput screening.

IV. MAINTAINING MOMENTUM

Much of the research I've highlighted is being conceived and conducted by scientists in laboratories and clinics across the country and at NCI - building on the wellspring of scientific discovery. Our goal for Fiscal Year 2003 is to speed the rate of discovery and translation of those discoveries to cancer patients by expanding and facilitating researchers' access to resources and new technologies. To understand the basic processes of cancer and translate this research into clinical practice, we must link researchers with the resources and technologies they need while encouraging multi-disciplinary collaboration.

NCI will continue to create and sustain research infrastructures for collaboration, technology support and development, and access to resources that enable multiple scientific disciplines to address the complex questions before us. We will achieve this by expanding our nationwide infrastructure of cancer centers, centers of research excellence, networks, and consortia in ways that promote and facilitate complex scientific interactions and the sharing of information and resources. Two important programs deserving of special mention are Rapid Access to Intervention Development (RAID) and Rapid Access to Preventive Intervention Development (RAPID). These programs expedite new agent development on the part of independent investigators in universities or biotechnology companies by making NCI's preclinical drug development resources and expertise available for moving novel molecules toward clinical trials.

Also key to our multidisciplinary approach are Specialized Programs of Research Excellence (SPOREs). Several major academic centers of excellence are now working on a wide range of scientific approaches to translational research - that is, focusing on the biology of cancer specifically as it may inform development of new treatments. NCI will expand the use of SPOREs in the coming year.

We will continue our efforts to ensure that the clinical trials program addresses the most important medical and scientific questions in cancer treatment and prevention quickly and effectively through state-of-the-art clinical trials that are broadly accessible to cancer patients, populations at risk for cancer, and the physicians who care for them. Despite major advances in our understanding of tumor biology and potential molecular targets for cancer prevention and treatment, our capacity to apply and test these findings in clinical settings has not kept pace. The NCI will invest more resources in developing and testing new therapies and increasing access to and participation in clinical trials. We will also expand surveillance data systems, methods, communications, and training to improve capacity for monitoring progress in cancer control and for exploring potential causes of cancer nationally and among diverse, underserved populations. NCI is also launching research to improve the quality of cancer care by strengthening the information base for cancer care decision making. Researchers must better understand what constitutes quality cancer care, with an emphasis on the patient's perspective; identify geographic, racial/ethnic, and other disparities in who receives quality care; and strengthen the scientific basis for selecting appropriate interventions. Finally, to sustain new ideas, we will continue to nurture and develop new scientists. To deliver new biology-based interventions, we must educate and train capable physicians. That's why NCI will continue to expand its efforts to design and implement opportunities for scientists at all career levels to meet the challenge of building a stable, diverse cadre of basic, clinical, behavioral, and population scientists trained to work together effectively and use the most advanced technologies.

V. CLOSING

NCI's mission is broad and our approach is necessarily ambitious, because, while our primary role and our expertise is research, our focus and sense of urgency is in serving the American people, the country's cancer patients and their families, friends and neighbors.

As director of NCI, a doctor, an investigator, and a cancer survivor, I share the urgency of America's cancer patients and I am confident that the efforts I've highlighted and many additional activities will bring us closer to the ending the death and suffering caused by this disease.

VI. BUDGET STATEMENT/GPRA

I am pleased to present the President's budget request for the National Cancer Institute for FY 2003, a sum of \$4,724,505,000, which reflects an increase of \$514,784,000 over the comparable Fiscal Year 2002 appropriation.

The NIH budget request includes the performance information required by the Government Performance and Results Act (GPRA) of 1993. Prominent in the performance data is NIH's second annual performance report which compares to our FY 2001 results to the goals in our FY 2001 performance plan.