

# **Testimony**

## **Before the**

### **Subcommittee on Labor, Health and Human Services, Education, and Related Agencies**

#### **Committee on Appropriations**

#### **United States Senate**

#### ***FY 2011 Budget Overview: National Institutes of Health***

##### **Statement of**

**John E. Niederhuber, M.D.**

**Director**

**National Cancer Institute**

**National Institutes of Health**

**U.S. Department of Health and Human Services**

For Release on Delivery  
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Mr. Chairman and Members of the Committee:

I am pleased to present the President's Fiscal Year (FY) 2011 Budget request for the National Cancer Institute (NCI) of the National Institutes of Health (NIH). The FY 2011 request includes \$5,264,643,000 for NCI, which reflects an increase of \$162,977,000 over the comparable FY 2010 appropriation level of \$5,101,666,000.

These dollars will support our efforts to deliver on the promise of a different kind of cancer care in this country – targeted, patient-centered care that is based on the individual genomic and

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biologic characteristics of patients and their tumors. These funds will also underpin the ongoing development of a system in which access to the latest science and care is available to all Americans, regardless of their means or locale. Today's possibilities for cancer research and the promise it offers are vast, and its potential impact on the lives of Americans is significant. The National Cancer Institute is committed to more quickly bringing better treatments to patients and to developing highly targeted cancer prevention and early detection strategies for all Americans.

For FY 2011, NCI seeks to enhance key programs and initiatives that could hasten our research progress against cancer. We have a tremendous opportunity to take a bold step into the future of cancer prevention, diagnosis, and treatment. Our efforts to devise streamlined and innovative approaches to clinical research and clinical trials; to bring the fruits of our science to patients in the communities where they live; to eliminate the all-too-common inequities in cancer care; and to invest in supporting infrastructure will make this new era of personalized, highly prescriptive cancer medicine possible.

### **Deeper Understandings Of Cancer's Inner Workings**

Cancer is an extraordinarily complex disease of uncontrolled cellular growth, proliferation, and spread, combined with unique networks of chemical interactions between tumor and the host. Cancer is not singular in definition. It differs according to the organ site of origin and often has important genetic and physical subtype differences. Our understanding of the inner workings of the cancerous cell and the body in which it resides are accelerating at an unprecedented pace, providing new opportunities for intervention and prevention. Genomics is contributing—virtually on a daily basis, it often seems—to our catalogue of knowledge about the mutations, genomic alterations, and biologic processes of cancer. In an era when molecularly targeted cancer therapies are an accumulating reality, the challenge we face is to turn groundbreaking science into lifesaving care, at an even greater speed.

In 2005, NCI and the National Human Genome Research Institute launched the Cancer Genome Atlas (TCGA), a large-scale, multi-institutional effort to sequence and characterize the changes (both genetic and epigenetic) associated with the development of cancer. In its pilot phase, TCGA set out to sequence the genomes of three cancers: glioblastoma (the most common form of brain cancer), lung cancer, and ovarian cancer. In glioblastoma, to take just one example, TCGA has identified four distinct subtypes of the disease that will help to stratify patients into different treatment regimens. Before, we saw glioblastoma as one disease - patients were all treated with drugs that had shown benefit for glioblastoma. Those drugs only worked in a percentage of patients, but we had no scientific way to predict which patients would benefit. Now, we know that there are four subtypes, and this will enable us to identify targets, develop drugs, and prescribe treatment for patients according to a particular subtype of glioblastoma. Because of TCGA's enormous potential, we are expanding the focus of TCGA to explore approximately 20 additional tumor types in the next two years.

## Taking The Genome To The Clinic

TCGA, along with other investments in genomic study, is the engine of a coordinated platform that will begin with new genomic discoveries and lead to new therapies. NCI's focus is to develop a comprehensive approach to translate raw genetic information into an intimate understanding of the function of the genetic pathways which can then be used to clearly define targets for manipulating those pathways.

Moving from data, to function, to target, to therapy will not be simple nor will it be easy. It is, however, possible and NCI is initiating a number of key programs that will link the genomics of cancer with new ways to diagnose and treat the disease. The first step will be to ensure the availability of high-quality human biospecimens for cancer research, accomplished through the cancer human biobank (caHUB). Patient Characterization Centers will further analyze patient's tumors and the Chemical Biology Consortium will develop and screen compounds aimed at specific targets. NCI's Experimental Therapeutics and Accelerating Clinical Trials Programs will test new compounds in patients. And throughout the process, information will be managed through the cancer Bioinformatics Grid (caBIG).

**Biospecimens** The cancer Human Biobank (caHUB) will be a national biobank – a repository for difficult-to-obtain biological materials and associated data that can be used for medical research. These samples, all rigorously and ethically collected, properly stored, and extensively annotated, will be used to develop new approaches for cancer diagnostics and drug development.

**Patient and Tumor Characterization** As we learn more about the specific genetic alterations associated with disease, cancers will be diagnosed and treated based on their individual molecular make-up. The molecular characteristics of patients and their tumors, both primary and metastatic, therefore will be more clinically significant than the organ site where the cancer originated. NCI's planned Molecular Characterization/Clinical Assay development center and Patient Characterization Center will be part of a small national network of centers that will bring together genomics and genetics, proteins and proteomics to develop personalized, highly prescriptive cancer care, matching the patient to tailored interventions.

**Biologic Function and Target Identification** Probing cancer's complex networks of signaling pathways requires cutting-edge chemical tools, which often exceed the capacity of an individual laboratory or an individual research university. The Functional Biology Consortium will be constructed as a virtual network of investigators who take promising genetic alterations identified through TCGA and other efforts and answer specific questions about the biologic function and potential drugable targets. Along with NCI's functional biology efforts, the Chemical Biology Consortium (CBC) is comprised of hundreds of scientists working to increase the flow of early-stage drug candidates into the developmental pipeline. The CBC will provide the necessary chemistry to determine early on which compounds hold promise and should be moved into pre-clinical testing and toxicology.

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**Drug Development and Early Phase Clinical Research** NCI has consolidated its anticancer drug discovery and development resources in support of a robust, balanced, goal-driven therapeutics pipeline. Combined, these resources are capable of supporting a discovery and development continuum from initial discovery through Phase II clinical trial evaluation. The NCI Experimental Therapeutics Program (NExT) will coordinate the many back-and-forth transitions that must occur between academic research laboratories, NCI, and the private sector. This will shorten the typical 10 to 12 year drug development timeline by up to six years, getting promising drugs into human trials more quickly—and more rapidly eliminating drugs unlikely to be effective. Through the Accelerating Clinical Trials of Novel Oncologic Pathways (ACTNOW) initiative, NCI funded 37 new Phase I and Phase II cancer treatment trials to test the effectiveness of molecularly-targeted cancer therapies in 2009. ACTNOW studies, which are truly prototypes for further studies in the years ahead, integrate the latest imaging technologies and correlative laboratory research studies, to help us understand the underlying biological mechanisms of action. Importantly, ACTNOW studies are on a strict, accelerated timeline which requires investigators to finalize institutional review board approval and begin enrolling patients within 90 days. This is far faster than current procedures.

**Bioinformatics** Through a single infrastructure, with standard rules and common language, the cancer Biomedical Informatics Grid (caBIG) builds and disseminates information and software tools, including the implementation of electronic health records. Through the development of a centralized repository of cancer care encounters, NCI is looking to establish a virtual cohort comprised of people at risk for cancer, patients with active disease, cancer survivors, and patients with recurrent disease. This cohort will enable individuals to work with their health care provider to determine appropriate treatment options for their disease; allow community oncologists access to optimal care solutions for cancer with specific genetic characteristics; and provide researchers with the necessary information to design clinical trials that match patients, based on their genetic make-up, to specific research efforts. Such a cohort will expedite research and improve cancer care, saving time, money and ultimately lives.

## **Putting Science To Work For Patients**

From genomics, proteomics and systems biology to efforts to reduce cancer risk for all patients, the Institute remains committed to every avenue of cancer research, be it laboratory, clinical, or behavioral. Through its network of NCI-designated Cancer Centers, as well as through programs such as the NCI Community Cancer Centers Program (NCCCP), the Specialized Programs for Research Excellence (SPORE), and the Community Clinical Oncology Program (CCOP), NCI is dedicated to expanding cancer research and delivering the latest, most advanced cancer care to all Americans.

Thank you for the opportunity to provide you this testimony. I look forward to the opportunity to take your questions.

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## **John E. Niederhuber, M.D.**

### **Director, National Cancer Institute**

John E. Niederhuber, M.D., became Director of the National Cancer Institute (NCI), in September 2006. He joined NCI in a full-time capacity in September 2005, as Deputy Director for Translational and Clinical Sciences, and within a few weeks was asked to serve as Chief Operating Officer. He officially became NCI's Acting Director in June 2006. Together with Dr. Francis Collins, he began the Cancer Genome Atlas, an effort to comprehensively identify the genomic changes in all major cancer types and subtypes. In addition to genomic studies of cancer and work in cancer immunotherapy, programs in nanobiology, systems biology, investigations into the tumor microenvironment, cancer initiating cells, and subcellular imaging have benefited under his direction.

Dr. Niederhuber is recognized by his peers as a visionary leader in oncology. His colleagues have acknowledged his leadership and accomplishments by electing him vice president and president of the Society for Surgical Oncology and president of the Association of American Cancer Institutes. He has served as a member of C-Change (a community of executives from government, business, and the non-profit community dedicated to conquering cancer) and as a member of the CEO Roundtable on Cancer. Dr. Niederhuber is a member of the Institute of Medicine of the National Academy of Sciences, recognizing his outstanding scientific accomplishments and commitment to service in health sciences.

As a surgeon, Dr. Niederhuber's clinical focus has been on gastrointestinal cancer, hepatobiliary (liver, bile duct, and gallbladder) cancer, pancreatic cancer and breast cancer. Recognized for his pioneering work in hepatic artery infusion chemotherapy, he was the first to demonstrate the feasibility of totally implantable vascular access devices which dramatically changed the administration of systemic chemotherapy.

Prior to coming to NCI, Dr. Niederhuber was Director of the University of Wisconsin Comprehensive Cancer Center and a professor of surgery and oncology (member of the McArdle Laboratory) at the University of Wisconsin School of Medicine. Earlier in his career, he chaired the Department of Surgery at Stanford University and held professorships at the Johns Hopkins University School of Medicine and at the University of Michigan.

A native of Steubenville, Ohio, Dr. Niederhuber is a graduate of Bethany College in West Virginia (receiving an honorary doctorate in 2007) and the Ohio State University School of Medicine. He trained in surgery at the University of Michigan.