Written Testimony to the United States Senate Committee on Health, Education, Labor and Pensions Regarding Cancer: Challenges and Opportunities in the 21st Century Submitted May 8, 2008

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On behalf of Dana-Farber Cancer Institute, an NCI-designated comprehensive cancer center located in Boston, Massachusetts, thank you for inviting me to testify at today's hearing on comprehensive cancer legislation. As a comprehensive cancer center director, I, and the colleagues and patients that I represent, have a deep interest in all aspects of the forthcoming cancer legislation. My distinct role today, however, is to reflect on the essential need for fundamental and applied cancer research. I have had the privilege to serve as the co-chair of a recently-formed Research Working Group, a panel of physicians, scientists, advocates and policy specialists convened to provide expertise and formulate recommendations to revolutionalize the cancer research enterprise. We appreciate the chance to share those recommendations with you now.

A Vision of the Future of Cancer Care

The world of cancer care is changing before our eyes. The era when treatments were focused on the organ where a cancer originates is coming to end. In the not-toodistant future, patients may receive therapies geared to the specific molecular characteristics of their disease. These customized treatments could include agents able to block the particular genes and proteins that have gone awry in the cancer tissue. Such agents will be supplemented by others that choke off the blood supply to tumors, limiting their size, and by vaccines that mobilize the body's natural immune defenses against cancer. Still other agents could take aim at the tumor's ability to spread to other parts of the body. The effect of such treatments could be tracked by imaging technology capable of showing, in precise detail, the extent of death of tumor tissue.

Other changes might be just as dramatic. The same knowledge that would enable us to halt the genetic machinery of cancer could lead to agents that can prevent cancer in people at risk for it. We'll hope to have a better handle on why some populations — for genetic, cultural, or economic reasons — have a greater likelihood of getting cancer and lower rates of successful treatment. We expect to know the safety issues associated with each form of treatment and have effective protocols for minimizing them. And we'll ensure that the environment in which patients are treated – hospital, clinic, or home — is as responsive to patients' needs and well-being as possible.

Ambitious as all this might sound, the fact is, some elements are already in place, and more are coming on line every year. The completion of the Human Genome Project has spurred the development of several "targeted" therapies that take aim at specific malfunctioning or misbehaving genes. The best-known of these are Herceptin[®], which has benefited thousands of women with a specific type of breast cancer, and Gleevec[®]. which is now the standard of care for many patients with chronic myelogenous leukemia and the digestive tract cancer known as gastrointestinal stromal tumor (GIST), for which there previously was no effective therapy for many patients. Blood vessel-blocking drugs known as angiogenesis inhibitors, such as Avastin[®], have become part of the regular arsenal of therapies against several kinds of cancer, including colon cancer. In recent weeks, a study has found that in patients with metastatic melanoma — a condition for which no effective treatment exists — Gleevec can drive the disease into remission if the cancer cells contain a key genetic mutation, or abnormality. These optimistic projections for the future could only happen if we are able to build on the research momentum generated by the human genome project and other advances, which will only happen if research funding growth is restored to at least its historical pace.

The Many Forms of Research

The groundwork for all these advances has been laid by an unprecedented degree of research — most of it government-funded — at academic and private institutions across the United States and overseas. A great deal of this exploration has occurred at the level of basic science — in which investigators study the fundamental workings of normal and cancer cells – and clinical science — where potential therapies are tested in human patients — but this represents only a portion of the full spectrum of cancer research. Equally robust efforts are under way in the areas of cancer prevention, patient safety, quality of care, quality of life, nursing, health disparities, and treatment outcomes. Much of this work necessarily takes place in health centers, but much is done in cooperation with community groups such as employers, religious organizations, tenants' groups, and neighborhood associations.

The reason for this broad focus is that cancer is truly a multi-dimensional problem — first and foremost, a matter of individual health, but one that affects people's loved ones, finances, occupation, education, and community, and one that reverberates on a local, state, and national level. Just as cancer needs to be attacked biologically on a variety of fronts, so does cancer research need to concern itself with all the implications of the disease and its treatment. We will not be able to truly to defeat cancer unless we grapple with the entire array of issues associated with the disease.

Cancer's Continuing Toll

Despite significant and steady gains against cancer — seen most clearly in a slow but uninterrupted decline in U.S. cancer death rates over the past three years — the disease continues to take a devastating toll. In 2008, there will be 1.44 million new cases of cancer in the United States (not including more than one million new cases of basal and squamous cell skin cancer) and an estimated 565,650 cancer-related deaths, according to the American Cancer Society. The number of new cases, which stood at 1.25 million in 2002, is rising each year as the American population ages. Nor are the physical, emotional, and financial costs of the disease spread evenly across the population: the National Cancer Institute states that the burdens of cancer are "unfairly shouldered by the poor, the elderly, and minority populations." Financially, the annual bill for cancer care in this country exceeds \$200 billion.

Laying the Foundation

Clearly, an immense amount of work remains before cancer can be declared "conquered." Research over the past two-plus decades has provided a scientific and social foundation from which we as a nation can launch a truly decisive assault on the disease. We know in intricate detail the genes and combinations of genes that cause tumors to form and drive their growth. We know, with equal specificity, the body's responses to the formation and spread of cancer. We have devised ways, in many instances, of blocking these genetic malefactors and the proteins they're responsible for – including the use of sub-microscopic nanoparticles or lab-made proteins that home in on key genes and stifle their activity.

In other facets of the cancer riddle, researchers have developed effective communication techniques and public-service campaigns for informing people – at home, on the job, where they shop, and where they go to school — about how to reduce the risk of cancer. Hospitals have designed systems for ensuring that when patients are treated for cancer, they're treated in the safest possible environment with powerful safeguards against medication errors. Investigators are compiling examples of "best practices" — determining which treatment approaches are most successful and advocating for them to become the standard of care. Other scientists are cataloguing the ways that diet and behavior influence people's risk of developing cancer. Still others are charting racial, ethnic, and socioeconomic disparities in people's risk of contracting cancer and their likelihood of receiving proper treatment for it.

The cumulative effect of this work — in the lab, the clinic, and the community — is to place the nation's cancer research enterprise on the brink of dramatic gains against the disease in the years ahead. In many respects, the work undertaken thus far can be viewed as a down payment on the new generation of therapies now taking shape.

Areas of Focus

In surveying the state of cancer research in the United States, the Research Working Group has identified a number of problem areas that are impeding optimal progress. Our recommendations offer ways of rectifying those problems and reinvigorating the nation's overall cancer research effort. We have divided our study into seven broad categories, which we summarize below.

I — Translational Research

The National Cancer Institute-supported effort to convert basic scientific findings into new and better therapies is not keeping pace with the advances in knowledge and technology over the past 40 years in cancer research. Among our recommendations to remedy this situation are: a special funding program to advance a select number of especially promising early research opportunities; joint NCI/industry funding of collaborative early translational research projects; and increased NCI interaction with foundations and advocacy groups to advance this type of research.

II — Clinical Research

Clinical trials are becoming increasingly complex to conduct, and the NCI's perpatient reimbursements are insufficient to cover the costs of such trials. Among our recommendations: additional Medicare payments to cover the additional time and resources involved in enrolling patients in trials; and group and individual health insurance mandates to cover the routine costs of participation in trials.

III — National Collection of Tissues/Biospecimens

Cutting-edge cancer research is impaired by the absence of either a centralized network of biospecimen and tissue collection banks, or consistent standards for retention and storage of such specimens. Among our recommendations: establishment of a National Cancer Biospecimen Network by linking existing public and private biospecimen and tissue collection banks; and guarantees of protections against genetic discrimination.

IV — Prevention and Early Detection Research

Despite the launching in 2000 of the Early Detection Research Network by the NCI, only a few biomarkers – substances in blood or other fluids that serve as telltale signs of cancer – are routinely used in oncology today. Discovery of new ones is hampered by the limitations of current technology. Among our recommendations: a standard process for developing, testing, and proving the value of biomarkers; support for high-quality biorepositories of samples of cancer sites; and federal and private health insurance coverage of new biomarker tests.

V — Young Investigator and Oncology Nurse Workforce

Teaching and mentoring the next generation of investigators is one of cancer scientists' most important jobs, but many of today's brightest young researchers are finding it increasingly difficult to establish independent careers in biomedical research and are leaving the field. Equally disturbing trends are threatening the vitality of the oncology nursing workforce, which is critical to quality care for patients. Among our recommendations: more stable funding streams to allow individuals and institutes to better plan projects and careers; more opportunities for non-U.S. citizens to emigrate and compete for training, postdoctoral and research awards; and fully funding for federal nurse loan repayment and scholarship programs.

VI — Collaboration

There is a lack of collaboration among NCI-funded cancer centers and programs, and a variety of barriers discourage partnerships between publicly and privately funded researchers. Pharmaceutical and biotechnology firms have little financial incentive to develop treatments for rare cancers. Among our recommendations: expansion of the Bayh-Dole Act to permit cancer-related partnerships between academia, nonprofit organizations, and private companies; and remove some restrictions on international sites that participate in NCI-funded trials.

VII — Federal Funding

Ten years ago, the nation made a bold, five-year investment in the National Institutes of Health and the National Cancer Institute, the primary federal vehicle for advancing cancer research. Between 1998 and 2003, NIH appropriations for cancer research essentially doubled, far outpacing the historic norm of 8.2% percent average annual increases. Since that period, however, the budget for such appropriations has been flat or declined. As the accompanying chart shows, had the five-year doubling never occurred and the 8.2% average been maintained each year since 1998, the appropriations budget would be significantly higher than it is today. Funding cuts for extramural research have been even more dramatic if one takes into account the allocations made for other NCI obligations. The result of this falloff is that many experienced researchers are struggling to obtain funding for more conservative, less-ambitious projects, while young investigators are increasingly abandoning the field. Without a renewed commitment to funding, the potential for new treatments, cures, and prevention strategies for cancer will continue to recede. Among our recommendations: consistent and sustained federal funding for research; support programs to improve the accuracy, completeness and accessibility of cancer data; and establish an office for rare cancers to ensure that research needs are met.

Conclusion

Decades of research have brought us to the point where some of the most dramatic advances in the history of the disease's treatment are coming into sight. The American public has made an investment in cancer research unequalled by that of any other nation, in the hope that such research will lead to better treatments and long-term cures. We have the opportunity, now, to honor that investment by ensuring a level of funding that will bring the promise of current cancer science to fruition.

The Research Working Group encourages the members of the Senate Committee on Health, Education, Labor and Pensions to provide the financial, regulatory, and legislative tools to carry the War on Cancer to its decisive stage. *****