NCI Cancer Research: Today's Progress; Tomorrow's Challenges

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Jeff Allen, Ph.D
Executive Director
Friends of Cancer Research

Introduction

Good afternoon, Chairman Pallone, Ranking Member Deal, Mr. Shimkus, and Members of the Subcommittee. I am Dr. Jeff Allen, Executive Director of Friends of Cancer Research, a cancer research advocacy organization and think tank based in the Washington area. I would like to thank the staff of this committee who have worked tirelessly in putting together this hearing. It is an honor to testify before you today on the importance of cancer research at the National Cancer Institute (NCI) and across all sectors. My testimony is intended to give perspective on the vital need for NCI to be in direct collaboration with other federal health agencies, the important role that public-private partnerships can play in spurring innovation, and the need to tear down the silos that currently exist within the biomedical research community.

The founder of Friends of Cancer Research, Dr. Ellen Sigal, could not be here today, as she is with a loved one right now that is being treated for a rare cancer. Dr. Sigal started this organization 15 years ago after having lost a sister to breast cancer, her father to prostate cancer, and mother to pancreatic cancer. This is as personal for her, as it is for you Mr. Chairman, and likely everyone in this room, including myself, who have been deeply affected by this terrible disease. It is with this in mind that I am here today to express what we feel needs to be done to end the suffering that millions of cancer patients and their families experience every year.

Nearly forty years ago President Nixon declared war on cancer by signing *The National Cancer Act* (P.L. 92–218) into law, and said, "The time has come in America when the same kind of concentrated effort that split the atom and took man to the moon should be turned toward conquering this dreaded disease. Let us make a total national commitment to achieve this goal."[1] Four decades later, we also have a President that has committed to ending cancer in our lifetime.

Over the past forty years, exceptional progress has been made in the treatment of cancers, due in large part to the investments in biomedical research. This has allowed for an understanding of the underlying biology of the disease in much more scientific detail. For example, since the declaration of a war on cancer, blood cancers have gone from being classified as five different types of leukemia and lymphomas to nearly 90 different disease subtypes based on biological characteristics. This has lead to the development of more tailored treatments that are more effective and in many cases less toxic. The current five-year survival rates for leukemia and lymphomas now average upwards of 70%.[2] That is at least twice, and in some cases, four times what they were in 1970.[3]

While this type of progress is compelling and observed in a few other cancers as well, there is much more to be done to alleviate the burden of cancer. It is estimated that, in 2009, nearly 1.5 million Americans will have been diagnosed with some form of cancer. As a result, our healthcare system will be strained an additional $228 billion.[4] Most importantly, this disease will claim the lives of 562,340 mothers, fathers, grandparents, sisters, brothers, and friends, per year.[5]

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[1] Remarks by the President in State of the Union Address: January 22, 1971 http://www.c-span.org/Content/HTML/Executive/Transcripts/nixon1971.pdf Accessed 3/19/10

[2] Allison, M: *Is Personalized Medicine Finally Arriving?* Nature Biotechnology. Vol. 26; No. 5, May 2008: 509-17.

[3] Facts 2009-2010. The Leukemia & Lymphoma Society. http://www.leukemia-lymphoma.org/attachments/National/br_1247234696.pdf Accessed 3/19/10

[4] The American Cancer Society: http://www.cancer.org/docroot/MIT/content/MIT_3_2X_Costs_of_Cancer.asp Accessed 3/19/10

[5] NCI Surveillance, Epidemiology and End Results: http://www.seer.cancer.gov/ Accessed 3/19/10

Biomedical research is an enterprise that is built on the benefits of capitalism, and all sectors gain from and are required for its success. Today, it is estimated that it requires over $1 billion, 12 to 15 years, and thousands of patient volunteers to get a single drug to market. Fifteen years to translate a new discovery to a therapeutic treatment, by today's rates, results in the loss of almost 8.5 million Americans approximately the population of state of New Jersey.[6]

Let us join together and commit that hearings like this, which are so important to refocus on these issues, will not end with a few comments for the record, but will actually bring about new action. There is an ever growing need for bipartisanship, to not let political posturing hinder the progress we have made as we strive to discover new life-saving treatments and therapies.

The strides forward in cancer research have been large, but due to the complexity of the disease, new multi-institutional approaches must be fostered. The funding allocated to biomedical research as a part of the American Recovery and Reinvestment Act of 2009 (ARRA) presented a renewed opportunity for American investigators to carry out research projects that otherwise may not have been possible. The programs supported by the National Caner Institute (NCI), from both the ARRA and the traditional budget process, continue to advance cancer research. However, a sustained funding commitment for biomedical research is needed to build on our prior investments and see that the returns on those investments reach the clinics and benefit the millions of patients, as they were designed to do.

Sustained funding is just one component to reducing the cancer burden.

In order to truly halt the devastating impact of cancer, a comprehensive approach is needed. The NCI and its programs are an engine that drive progress, but increased collaboration with other entities, both public and private, are needed to turn the next corner of scientific advancement.

We must also acknowledge the need for a streamlined process in getting the discoveries being made at the NCI to patients, as safely and efficiently as possible.

To accomplish this Congress needs to support increased scientific capacity of the Food and Drug Administration, which serves as the nexus between the progression of laboratory research and the clinical use of new therapies.

We ask that you support President Obama's fiscal year 2011 budget request which contains a much needed 6% increase to the budget authority for the FDA, including $25 million allocated to Regulatory Science, which aims to develop, assess and provide new, validated tools and approaches to better evaluate the utility of new medical products. This initiative has been spearheaded by the visionary leadership of FDA Commissioner Dr. Margaret Hamburg.
The success of the NCI cannot be measured solely as the research accomplishments facilitated by the institute, but rather by its contribution to the larger goal of reducing the national cancer burden, and this cannot be achieved alone. It is critical to also examine the impact of other federal agencies on the ability for NCI-based discoveries to ultimately improve the lives of patients. For example, in 2007 the Food & Drug Administration (FDA) Science Board declared the agency's "mission at risk" due to its eroded scientific

[6] United States Census Bureau: http://factfinder.census.gov/servlet/SAFFPopulation?_submenuId=population_0&_sse=on Accessed 3/19/10

foundation.[7] Clearly, without a scientifically rigorous regulatory body, discoveries facilitated by NCI-based research could be inefficiently or inappropriately evaluated, and ultimately not achieve the envisioned improvement to patient's lives.

Even prior to the report that highlighted the need to advance the science of regulation, the FDA acknowledged the need for assembling oncology expertise at the agency through the establishment of the Office of Oncology Drug Products.[8] While this has made several improvements to the regulation of oncology programs, a comprehensive FDA Oncology Program is still needed to facilitate and increase the transparency of intra-agency collaborations, standardize review guidelines, and establish jurisdiction and sufficient interactions between FDA Centers that are frequently involved in the review of increasing complex new product applications. A robust cancer program can also build upon existing collaborations in order to increase the scientific methodologies used by the agency.

The NCI knows the power of such collaborations first hand through the development of the NCI-FDA Interagency Oncology Taskforce (IOTF).[9] This has allowed for new training mechanisms and an exchange of ideas that capitalizes on the great expertise at both agencies in order to efficiently translate NCI-based discovery to patient benefit. However, with additional resources the IOTF could expand its current portfolio to include additional scientific programming. It is in that spirit that on February 24th this year, the leaders of the National Institutes of Health (NIH) and the FDA announced the formation of the Joint Leadership Council in order to take the necessary steps to work together to advance the missions of each respective agency.[10]

It is these types of collaborations that are needed across the federal health agencies to streamline cancer research, detection, treatment, prevention, surveillance, product regulation, care delivery, reimbursement of services, and to learn from all of these cancer-related functions as a routine by-product of care. This will ultimately lead to increased survivorship, reduction of cancer incidence and mortality, and improvement of the lives of all who may face this disease.

The collaboration cannot occur within federal government agencies alone. Public-private partnerships, like that of the Foundation for the NIH or the Reagan Udall Foundation for the FDA, are the direction that we must go in order to capitalize on all research being conducted around the world.

Last week, the Biomarker Consortium, a public-private partnership through the Foundation for the NIH, announced the opening of the I-SPY2 TRIAL. This clinical trial will utilize an innovative new model that use biomarkers from individual patients' tumors to screen promising new treatments for breast cancer and identify which treatments are most effective in specific types of patients. This will allow researchers to use early data from one set of patients to guide decisions about which treatments might be more useful for patients later in the trial, and eliminate ineffective treatments more quickly. Included in this public-private collaboration are NCI, FDA, the Center for Medicare and Medicaid Services (CMS), the Pharmaceutical Research and Manufacturers of America (PhRMA),

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[7] Report of the FDA Subcommittee on Science and Technology: FDA Mission at Risk. November, 2007: http://www.fda.gov/ohrms/dockets/AC/07/briefing/2007-4329b_02_01_FDA%20Report%20on%20Science%20and%20Technology.pdf Accessed 3/20/10

[8] FDA News Release, July 16, 2004: http://www.fda.gov/NewsEvents/Newsroom/PressAnnouncements/2004/ucm108326.htm Accessed 3/20/10

[9] NCI-FDA Interagency Oncology Taskforce: http://otir.cancer.gov/programs/partnerships_iotf.asp Accessed 3/20/10

[10] United States Department of Health and Human Services: http://www.hhs.gov/secretary/speeches/sp20100224.html Accessed 3/19/10

the Biotechnology Industry Organization (BIO), major pharmaceutical companies, and numerous non-profit medical research organizations.[11] It is through collaboration that true progress will continue to be made.

In addition to fostering multi-agency and multi-institution collaboration, we must address the barriers to advancing the work achieved through NCI programs by modifying current policies. This includes the need for the streamlining of intellectual property agreements between academia, government, and industry, modifications to the HIPPA privacy rules that ensure patient protection but don't stand in the way of research, and data collection.

The historic health reform bill passed this week, developed by this committee and others takes many important steps to aid cancer research and ensure that breakthroughs from research are available and accessible to all Americans. We applaud the protections it provides for patients by prohibiting insurance discrimination due to a pre-existing condition. The provisions that safe guard the coverage of routine costs of care for patients that participate in a clinical trial will undoubtedly allow more cancer patients to participate in clinical research as an option of their care. This is a critical step forward in alleviating the challenges created by the currently low levels of participation in oncology trials. Numerous steps were also included to help provide much needed access to important tools for prevention, the ultimate defense against cancer. However, we must remain steadfast in our research support to develop even more preventive measures for the many cancers that we have no current means to prevent.

Another component of the health reform bill that Congress should be commended for addressing is the expansion of comparative effectiveness research (CER) programs. Additional support for CER can help to provide improved information for patients and their healthcare providers as they decide the best course of treatment. A focus on strengthening the information technology (IT) infrastructure in this country will help generate additional evidence to inform health outcomes research and CER.[12] In fact, the development of large-scale data networks will create an expansive collection of outcomes data for which comparisons of different treatment options can be performed. Furthermore, the use of harmonized data networks will help increase transparency to research priorities and generate further hypotheses for clinical studies based upon subpopulation characteristics, which in turn, will help to further advance "personalized" medicine.

In order for these activities to be successful we will need the previously described agency collaborations to develop research priorities, design appropriate study methodologies, identify of the most appropriate agency/organization to conduct the CER studies, as well as provide the resources necessary.

The NCI should be at the center of this type of work, and already has significant expertise in the development of data networks for cancer research that can support the development of more personalized medicine as well as be utilized or serve as a model for other health areas.

To begin to address the challenges associated with translating early phase research to clinically meaningful treatments, the health reform bill establishes the Cures Acceleration Network. This provision aims to address areas of high medical need and support future

[11] I SPY 2 TRIAL (**I**nvestigation of Serial **S**tudies to **P**redict **Y**our **T**herapeutic **R**esponse with **I**maging **A**nd mo**L**ecular Analysis 2): http://ispy2.org/ Accessed 3/19/10
[12] Improving Medical Decision Making Through Comparative Effectiveness Research: http://focr.org/files/CER_REPORT_FINAL.pdf Accessed 3/19/10

innovation in such areas by providing directed funding and streamlining processes required for the development of a potentially beneficial medical product.

These provisions in the health reform bill hold great promise for the future health of America. While it will take careful thinking to finalize many of the details moving forward, we look forward to working with members of this committee and others through the implementation process to ensure the success of these programs.

We must also tear down the silos that exist in biomedical research and focus on how all sectors can work toward the common goal of reducing the cancer burden.

Advances in cancer research have redefined how cellular abnormalities associated with cancer development and progression are studied and treated. Basic research findings have revealed that unregulated cell growth is often the result of altered signaling mechanisms that frequently involve multiple, complex molecular signaling pathways. This phenomenon is observed in many different cancers no matter what their tissue of origin may be. While the specific abnormality or mutation may likely be different in different cancers an improved understanding of the biology of cancers has identified potential commonalities that, when identified, may allow effective treatments to be utilized in multiple cancers that have been historically categorized differently based on their tumor site.

Classifying and studying cancers based on their molecular characteristics as opposed to just their tumor site is in many cases the direction that science is leading. This alternative approach in classification of different cancers has, and will continue to parse the number of people that actually have the same form of disease. Much like the subtypes observed in blood cancers, similar subpopulations are being defined in other cancers. For instance we now understand that "breast cancer" is a collection of hundreds of molecularly defined different diseases. This causes many more cancers to actually be "rare" diseases than originally thought.[13] Attention to this growth in the overall number of rare disease has been shown by NIH Director, Dr. Francis Collins, through his exemplary leadership of the NIH effort to develop new therapeutics for rare and neglected diseases.[14]

The identification of multiple signaling pathways and their molecular components involved in the complexity of cellular communication has created hundreds of targets for potential new treatments. In fact, several effective cancer treatments have been shown to provide great benefit in multiple cancer types that may have common characteristics. For example, both imatinib (Gleevec®) and bevacizumab (Avastin®) target specific cellular components and have been shown to be effective in different types of cancers that may have historically thought to be very different from one another. It is through the success of research that these common molecular targets responsible for abnormal growth have been identified in multiple cancers. Many experts speculate that the future of cancer research and treatment will involve the combined use of multiple targeted agents that are developed and utilized based on the analysis of tumor characteristics. While this presents numerous challenges to developing, regulating, and utilizing such a treatment approach, it provides a great hope for the future of cancer research.

The beneficial effect of the combination is often greater than the sum of its individual parts. This will hopefully hold true for the future of cancer therapies themselves, but the

[13] A rare disease is defined as a disease that affects fewer than 200,000 people in the United States. *The Orphan Drug Act of 1983* (P.L. 97-414)
[14] NIH Press Release, May 20, 2009: http://www.nih.gov/news/health/may2009/nhgri-20.htm Accessed 3/20/10

principle will also need to be applied by all the components of the cancer research enterprise if we are to accelerate the pace to reduce the burden of cancer.

In order to create a synergistic effect in cancer research, existing institutional barriers will need to come down with a renewed focus on the common goal. While currently it is true that the health burden due to cancer can be seen greater in different diseases, taking from one to give to another is not an effective strategy. This is not to diminish the important work and targeted focus, but we must let our work support and inform those fighting for the common goal on an alternative front.

The field of cancer research has built an unprecedented infrastructure that needs to be further capitalized on. The NCI currently has 65 designated cancer centers that should encourage both internal and inter-center coordination of research.[15] Philanthropic institutions have facilitated great breakthroughs through goal-oriented funding. Tension continues to exist between individual initiated research and large scale science, often leading to difficult decisions of supporting one over another. In reality, we need support for all types of research with increased accountability, clear targeted goals, enhanced collaboration, and support for one another's efforts.

The time of scientific opportunity is upon us. In order to ensure that the multiple, integral components of the health care system are prepared for the future of cancer research and treatment we must act now. In order to ultimately and successfully reduce the burden due to cancer, a renewed and unified commitment is needed – including researchers and caregivers, government officials and congress, industry and payors, patients and advocates. It is time for a new, unified approach to combating cancer as a whole.

Conclusion:

I cannot emphasize enough the need for collaboration. The advocacy community, and entire research community, must embrace our common goal and support science and collaboration that will enhance the battle against cancer on all fronts. It is our responsibility to represent patient's needs, and what is needed to end the burden of all diseases.

We respectfully ask that the members of this committee, the Congress and the Administration, be steadfast in their commitment to ending cancer, through not only sustained funding research but supporting and facilitating inter-agency collaboration, regulatory science, tearing down the silos, and encouraging the development of public-private partnerships.

[15] NCI Designated Cancer Centers: http://cancercenters.cancer.gov/cancer_centers/index.html Accessed 3/20/10