

Testimony for the Record
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For Hearing on
Promising Initiatives and Innovations for Cancer Cures and Treatments

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Chairman Chaffetz and Ranking Member Cummings, thank you for the opportunity to submit testimony on this timely and vitally important subject.

I commend you for your leadership and dedication to the promising initiatives and innovations that are already turning into durable treatments for patients with cancer. Decades of investment in cancer and biomedical research by our Government is paying off, and it's an exciting time. New treatments are being approved rapidly that are turning previously deadly cancers into chronic diseases that allow patients to live long and productive lives. Your efforts help research work for all Americans.

Scientists in the United States lead the world in cancer research innovation and success, and continued investment will relieve cancer suffering for all Americans and hopefully prevent cancer development in our future generations. I will focus on four key areas that underscore the importance of supporting cancer research: benefits of federal funding, our current challenges, creating collaborations and training the next generation of scientists.

I am the Deputy Director of the Sidney Kimmel Comprehensive Cancer Center at Johns Hopkins which is an NCI designated Cancer Center. Our mission is to reduce morbidity and mortality from cancer through research. Funding from the NCI gives Cancer Centers like ours the ability to support our scientists' research efforts, maintain and build core resources to develop new therapies, bring newly-developed therapies from the laboratory into clinical practice through clinical trials, and disseminate new scientific knowledge to the greater cancer community and the public. Many cancer centers like ours also work to reduce disparities in cancer care.

I am also the Dana and Albert "Cubby" Broccoli Professor of Oncology. I serve as Associate Director of the Bloomberg-Kimmel Institute for Cancer Immunotherapy at Johns Hopkins, and the Co-Director of the Skip Viragh Center for Pancreatic Cancer Clinical Research and Patient Care. In addition to these responsibilities, I am a member of 3 NIH funded graduate programs (Immunology, Cell and Molecular Medicine, and Pharmacology) and dedicate efforts to training the next generation of scientists. I am an internationally recognized expert in cancer immunology and translational research, with specific expertise in the early development of

immunotherapies for breast and pancreatic cancers. I have served on numerous academic advisory boards and currently serve as Chair of the National Cancer Advisory Board. I also served as a co-Chair of the NCI Blue Ribbon Panel for the national Cancer Moonshot Initiative. I am now the current President Elect of the American Association for Cancer Research. In summary, my 25-year career has been dedicated to finding cures for cancer and training young scientists.

Benefits of federal investment in research

We are in the midst of a technological revolution that is providing new insights into human biology and cancer. We are amassing huge amounts of information and using it to transform how we approach cancer treatment and prevention. To bolster this progress, the Beau Biden Cancer Cures Act within the 21st Century Cures Act was enacted. It comes at a fortuitous moment in this advancement of knowledge, providing a critical investment needed for new cancer research and precision medicine. This investment is needed in addition to the ongoing NIH and NCI budget allocations, to accelerate in 5 years what would take 10 years to move new discoveries into treatments for patients with cancer. It is important to stress that any cuts made to the NIH and NCI budget will slow future discoveries and innovation and will take away from the potential success of the 21st Century Cures Act that was intended to enhance the current and future NIH and NCI budgets.

I co-chaired the Blue Ribbon Panel, which engaged the entire cancer community to identify 10 priority areas ripe for the development of cancer treatments and prevention tools. These recommendations are a roadmap that leverages new advances already made in cancer diagnosis, prevention and treatment as a result of the long-term investment by our government in basic and translational research. There are many examples of recent successes that are already reducing suffering and improving the lives of patients with cancer.

One example of this is a cancer treatment that uses the body's own immune system to kill cancer. In just the last six years, a variety of "immunotherapy" drugs have been approved by the FDA for 20 different metastatic cancers that include malignant melanoma, renal cell carcinoma, non-small cell lung cancer, bladder cancer, head and neck cancer, and Merkel cell carcinoma. Patients with these cancers would have died of their disease in less than a year and now are living years with good quality of life.¹⁻³ This is the direct result of decades of investment in understanding how the immune system works and sees cancer. Our understanding the immune system has also led to the development of vaccines that can prevent cancers that are associated with two common viruses, human papilloma virus and hepatitis B virus. These advances are saving American lives and the lives of people worldwide.⁴⁻⁵

The rapid pace of scientific discovery in how the immune system sees cancer has opened the door to new areas of research that would not have been possible even 5 years ago. As one example, the NCI is now investing in laboratory research and cancer screening studies to develop ways to prevent the majority of cancers that are not due to viruses. Most cancers develop as a result of stepwise genetic changes that transform normal cells into premalignant cells and then into cancers. Scientists are now working on developing vaccines that can recognize the earliest changes in the normal cell and eradicate these changes before they cause malignancies. This progress is possible because of government funding in basic research on the immune system, genomic sequencing research through The Cancer Genome Atlas, and the development of genetically engineered mouse models that utilize the genome sequencing data to emulate how cancers develop in humans.

Early development of vaccines for cancer prevention is already paying off. Even for a deadly disease such as pancreatic cancer, we can now use vaccines in patients who undergo surgical removal of their cancer to prevent the cancer from coming back. Federal funding was used to develop one such vaccine approach at Johns Hopkins. We have treated 60 patients with this vaccine in the earliest type of clinical trial that aimed to determine if it can be effective. These patients were expected to have recurrence of their cancer within 1 year. Instead, some of these patients are celebrating their survival more than 10 years later. If it weren't for federal funding, this study could not have happened and these patients may not be alive today.

Current Challenges

Although we are in a scientific revolution and making great progress, there are still many challenges to overcome. Several were identified by the Blue Ribbon Panel. One is the lack of progress that has been made for rare cancers. Among this category are pediatric cancers. Treatment for these cancers are not typically developed by the Pharmaceutical Industry due to their rarity. In contrast, the NCI has supported networks, including the Children's Oncology Group and Adult and Pediatric Brain Tumor Consortiums, that provide the opportunity for multiple cancer centers to work together to conduct research and clinical trials focused on these rare diseases. These networks are critical for progress yet they are underfunded and fall short of the more common cancers when it comes to developing new clinical trials that have the potential to bring new treatments to patients. One such NCI-funded network -- The Cancer Immunology Trials Network -- led to the development of an immunotherapy drug for a rare form of skin cancer called Merkel Cell Carcinoma. The FDA approved this therapy within a few years of when the Network began its clinical trials. This is historic progress for people with this type of cancer and could not have happened without a government investment in clinical research. We need to do more of this and more quickly to bring these new agents to patients with different cancer types.

The NCI is the main source of funding for research on pediatric cancers. Pediatric brain tumors are among the most difficult cancers to treat, and a rare type called diffuse intrinsic pontine glioma or "DIPG" is even more challenging. DIPG affects the brain stem, with cancerous cells interspersed with healthy brain cells. Due to its structure and location, surgery is not possible and it is almost always fatal. Over the past 10 years, research has provided an improved understanding of this cancer including the identification of genetic mutations that are potential targets for new therapies.⁶ These opportunities could only have occurred because of the Government's investment in this disease.

Another challenge is the need for enhanced population science research to identify barriers and provide solutions to people who typically lack access to the best cancer treatments. This research is needed for people in inner cities and rural areas, as well as for people of lower socioeconomic status, all of whom experience great disparities in cancer care. Government funded Cancer Centers provide the resources to conduct these population-based studies and offer solutions to overcome barriers to access. Johns Hopkins has made significant progress in the State of Maryland. Increasing minority access to care and recruitment into clinical trials has been a major priority for our Cancer Center. Maryland has more than twice the population of African-American citizens, compared with all 50 states. Cancer continues to affect minority populations disproportionately. Cancer death rates among black men are 27% higher than among white men, and 14% higher among black women when compared with white women.⁷ We have done significant outreach in the community to advance our understanding of factors that influence disparities, determine biologic differences that may contribute, and provide better care, including ensuring all populations have equal access to clinical trials. After studying the problem of clinical trials access among our own populations of cancer patients, we narrowed the gap between minorities and non-minorities who participate in clinical trials by 60% since 2001.

Maryland was once the state with the second highest cancer death rate. Those deaths have plummeted in our state, and we are now the 31st. This significant reduction is due in large part to Government funded screening programs that have been implemented in low income areas and our inner cities. These screening programs have identified individuals with early colorectal, prostate, breast, and cervical cancers and have provided them with earlier access to the best treatment and clinical trials. The US Centers for Disease Control and Prevention reports that Maryland excels in cancer screening, and this is due entirely to federal funding.

As our population changes we continue to face new challenges. Obesity and diabetes are increasing among young Americans. There are a number of cancers that are starting to increase in incidence as a result, including, liver and intrahepatic bile duct cancers. Continued government resources are needed to understand the science that links these chronic conditions to cancer development so that new screening approaches and interventions can be developed to intercept an impending cancer epidemic.

Collaborations

Collaborations between the NCI, the FDA, cancer foundations, advocacy groups, biotechnology and pharmaceutical companies, and patients is critical to ensure progress in reducing cancer morbidity and mortality. Government funding of early discoveries provides a foundation for new opportunities between academia, biotechnology companies, and pharma. These are important collaborations that could not occur without a federal investment in research because it is this investment that leads to the new innovations and early discoveries at the academic level that can then be further developed by biotech and pharma. We lead the world in technology development and research. We will only stay in the lead if we have increased government investment in the NCI and NIH.

The speed at which we are collecting data through cancer research makes collaborations and sharing of data critical. To make this data work well for us, we need to develop computational biology platforms for data processing and sharing. Our government stands poised to not only fund the generation of this data, but to also provide platforms for sharing it. The genomic sequencing

data generated by the federally-funded Cancer Genome Atlas is an excellent example. This data is used widely by researchers to generate genetic models of cancer development and drive the next questions in cancer biology and drug development. This investment has also led to a new area of medicine – precision medicine – which utilizes the genetics of a patient’s specific cancer to determine the best treatment. The NCI has since begun a national clinical trial called the MATCH trial which pairs patients with tailored options for clinical trials based on their tumor’s genetics.

The Blue Ribbon Panel identified data sharing as one of the top priorities. In addition to expanding the scientific data generated, the Panel recommended developing ways in which patients can access these data systems, input their own data for analysis by scientists, and also get “pre-registered” for clinical trials among all NCI-designated cancer centers. However, this recommendation can only be implemented with Government investment.

Next Generation of Scientists

The state of science and medicine are at a Crossroads. On one hand, we are in the middle of a revolution that is turning decades of government investment into real treatments that are saving lives. On the other hand, the perceived instability of government funding for research due to the threatened reduction in the NCI and NIH budget without significant increases in the past decade has created a crisis where young people are less inclined to pursue this as a career.⁸ The potential impact is grim – without the funding to launch a young scientist’s research in the US, our young scientists are turning elsewhere. They are taking positions outside academia, leaving the US for countries that are using sustained funding to recruit our young scientists, or they are leaving science entirely. The lack of sustained investments in research and training is leading to a severe reduction in the scientific work force and recruitment of the best and the brightest to the field. We need to take a lesson from history. Before the World Wars, Germany dominated research and technology development. Americans pursuing a career in science had to learn the German language to read their original research. Since then, the US has dominated science, technology and medicine. This was due to the sustained government investment over the past 70 years. Such an investment had many important outcomes including the deciphering of the biology of many diseases that led to new therapies and the rise of the biotechnology and pharmaceutical industry that developed these new therapies, created many jobs, and made the US leaders worldwide. We need to grow biomedical research to show young scientists that these are long-term careers.

Summary

Our worldwide dominance in cancer research has direct impact on people’s lives. It is this research that has given colon cancer patient Stefanie Joho a new life. At 23, Stefanie’s cancer had spread widely and she endured multiple regimens of chemotherapy. However, nothing worked. She was told she was inoperable and had no further options. She had debilitating pain and was down to less than 100 lbs. Stefanie’s sister was determined to help and scoured the internet. She found a clinical trial using an immunotherapy drug for some forms of colorectal cancer at Johns Hopkins, and the underlying scientific discoveries spanning several decades leading up to this trial were funded by the NCI. Within three days of starting the immunotherapy drug, Stefanie’s symptoms improved dramatically. Within 3 months, her tumor had shrunk 65%. More than a year later, she had a complete response. Stefanie remains healthy and is moving on with her life.

The recent successes in science and medicine cannot continue without an increased government investment. Now is the time to recommit this investment in science and medicine. A renewed and more robust investment will provide continued support for innovation, ensure the future health of our medical and technology industries, provide a sustainable career path for young scientists who will be the future innovators, and importantly, provide the opportunity to rapidly develop new cancer treatments and prevention strategies to once and for all eradicate cancer. Thank you again for the opportunity to speak to you today, and I look forward to answering any questions you might have.

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