

# Written Testimony to the Senate Appropriations Committee from the American Association for Cancer Research for the Hearing on "Driving Innovation Through Federal Investments" April 29, 2014

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The American Association for Cancer Research (AACR) is the world's oldest and largest scientific organization focused on every aspect of high-quality, innovative cancer research. The mission of the AACR and its more than 34,000 members is to prevent and cure cancer through research, education, communication and collaboration. We thank the United States Congress for its longstanding, bipartisan support for the National Institutes of Health (NIH) and for its commitment to funding cancer research. We especially thank Senate Appropriations Chairwoman Barbara Mikulski and Vice-Chairman Richard Shelby for their unwavering support for the NIH and for holding this important hearing on the federal investment in innovation.

#### **Investments in Cancer Research are Driving Innovation and Saving Lives**

Significant progress has been made against cancer because of the decades of federal investment in biomedical research and the dedicated work of researchers, physician-scientists, and patient advocates throughout the biomedical research enterprise. Federal support allows for innovative approaches to the prevention, detection, diagnosis, and treatment of cancer, and we have never been better positioned to capitalize on our hard-won understanding of what causes and drives cancer. This knowledge provides the foundation for innovative new treatments and preventive strategies, and is accelerating progress against the more than 200 diseases we call cancer. Federal investment in cancer research also leads to innovation in other fields. For example, drugs originally developed for cancer patients have led to treatments for macular degeneration, atherosclerosis, psoriasis, rheumatoid arthritis, and hepatitis among others.

As was detailed in the <u>AACR Cancer Progress Report 2013</u>, support from the National Institutes of Health (NIH) and the National Cancer Institute (NCI) of basic, translational, and clinical research has led to decreases in the incidence of many cancers, cures for a number of these diseases, and higher quality and longer lives for many individuals whose cancers cannot yet be prevented or cured. In fact, the number of cancer survivors living today in the United States is estimated to be more than 13.7 million.

Consider the progress made in just the last 18 months. Cancer patients now have access to:

- 13 new drugs to treat a variety of cancers
- 6 new uses for previously approved cancer drugs
- 3 new imaging technologies
- the first approval of a combination of targeted therapies for the same indication, and,
- the first high throughput sequencing machine, which will help tailor treatments for patients

The vigorous pursuit of new breakthroughs in cancer research and biomedical science is also serving as one of our country's primary paths to innovation, global competitiveness, and economic growth. According to United for Medical Research, NIH funding directly and indirectly supported more than 402,000 jobs in 2012 alone, and generated more than \$57.8 billion in new economic activity.

These remarkable achievements are a direct result of our national commitment to funding cancer research, screening, and treatment programs at the NCI, NIH, and other agencies across the federal government. We can continue to make significant advances—but only if we continue to allocate the required resources at the federal level to do so. Reversing recent cuts to the NIH and NCI and increasing funding for these agencies in fiscal year (FY) 2015 and beyond is essential to a full-scale national effort to lessen the burden of cancer.

This is a defining time in America's commitment to finally defeat cancer. We must seize the opportunity to invest our nation's remarkably productive biomedical research ecosystem by providing \$32 billion for the NIH in FY 2015. This investment will ensure we can continue to transform cancer care, spur innovation and economic growth, maintain our position as the global leader in science and medical research, and most importantly, bring hope to patients and their loved ones everywhere.

# Past Investments in Cancer Research are Driving Innovation and Yielding Dividends Today The nation's historical investment in cancer research is unquestionably having an impact, and we are seeing results in the form of clinical advances and new treatments. While the advances over the past few decades are too numerous to list here, breakthroughs in two areas—precision medicine and cancer immunotherapy—are of particular importance and are described in greater detail below.

### Precision Medicine is Re-shaping the Diagnosis and Treatment of Cancer

A significant milestone for cancer research was the discovery that cancer develops as a result of alterations in the genetic material of cells. Research in genomics has propelled technological innovations that are making it possible to efficiently read every known component of the DNA from an individual's cancer. Capitalizing on these advances is the goal of large-scale genomic enterprises such as The Cancer Genome Atlas (TCGA) and the International Cancer Genome Consortium (ICGC). These and similar initiatives aim to identify all of the genomic changes in many types of cancer by comparing the DNA in a patient's normal tissue with the tumor DNA, in order to discover the genetic alterations that drive a given cancer. This innovative approach, treating cancer based on the genetic and molecular profile of a patient's tumor, is often referred to as personalized, or precision, cancer medicine.

The success of precision medicine is exemplified in the development of drugs for patients with an aggressive form of breast cancer. More than 45,000 individuals will be diagnosed with a form of breast cancer that overexpresses the protein HER-2. Historically, outcomes for patients with HER-2 positive breast cancer were poor. But with the knowledge gained through genomic-based research and discovery, four HER-2 targeted therapies have been approved in the last 5 years, revolutionizing the treatment of the disease and bringing hope and significantly prolonged survival to tens of thousands of breast cancer patients.

As you can see, these discoveries are changing the way researchers view cancers, categorizing them more by the genetic changes that drive them and less by where they originate—in the breast, brain, lung, or liver, for example. It is also transforming the detection, diagnosis, and treatment of cancer. Although to date, large-scale genomic analyses have been completed for just a few types of cancer, with research into many others underway, the advances made are a step toward a future where most cancer treatment and prevention strategies are based on both a person's genetic makeup and the genetic makeup of their specific cancer. Had the federal government not made the wise decision to invest in mapping the human genome, none of this progress would have been possible.

### **Cancer Immunotherapy**

Another milestone for cancer research was the discovery that the immune system can identify and eliminate cancer cells the way it does disease-causing pathogens. The study of the structure and function of the immune system is a field of research called immunology and is more than 100 years old. Tumor immunology (sometimes called cancer immunology) is the study of interactions between the immune system and cancer cells.

Because the immune system naturally eliminates some cancers before they become life threatening, researchers thought that it should be possible to develop therapies that would train a patient's immune system to destroy their cancer. Such therapies, referred to as immunotherapies, are beginning to revolutionize the treatment of some cancers, such as metastatic melanoma and chronic lymphocytic leukemia by taking the brakes off or pressing the accelerator on the body's immune system, or by flagging cancer cells for destruction by the immune system.

While treating cancer by harnessing the body's own immune system is a dream dating back to the 1890s, tangible progress in this area has been recent. Until the first therapeutic cancer vaccine, sipuleucel-T or Provenge, was approved in 2010 for the treatment of prostate cancer, investigational immunotherapies were effective in so few patients that they did not become established treatment options. Today, with expanded knowledge thanks to basic and clinical research funded by the NIH, several types of immunotherapies have been approved and many more are in the pipeline. Researchers also are pursuing their use in combination with one another in the hopes of yielding even more positive results in patients in with cancers such as advanced melanoma and chronic myelogenous leukemia, among others. The success of immunotherapies in the past few years underscore how decades of research coupled with innovation in numerous disciplines are paying dividends for many cancer patients. Thanks to federal investment in research and the persistence and dedication of researchers across the biomedical research enterprise, the dream of immunotherapy is being realized.

## Cancer remains a significant public health challenge

Even in the face of the tremendous progress discussed above, cancer remains a formidable opponent. An estimated 1.6 million Americans will be diagnosed with cancer this year, and 1 in every 3 women and 1 in every 2 men will likely develop cancer in their lifetimes. It is also projected that more than 580,000 people will die this year in the U.S. from the disease, which is equivalent to 1,600 people each day. In fact, cancer will account for nearly one in every four deaths, making it the second most common cause of disease-related death in the United States. There also remain a number of cancers, including pancreatic, liver and lung cancers, for which the mortality rate remains extraordinarily high and 5-year survival rates are typically less than 50 percent. Further, racial and ethnic minorities, as well as low-income and elderly populations, continue to suffer disproportionately in cancer incidence, prevalence, and mortality.

Because of the steady increase in cancer incidence rates, which is mainly due to our increasingly aging population and the enormous complexity of many cancers, continuing and strengthening our nation's commitment to cancer research and biomedical science is more critical now than ever. Increasing the federal investment in cancer research and biomedical science will play a vital role in addressing the current challenges in cancer, while at the same time curbing the overall annual costs of this devastating disease —which exceeded \$263 billion in 2010, and the economic burden is expected to rise as the number of cancer deaths increases.

#### What is required for continued innovation and progress against cancer?

As we look to a future in which cancer care will be transformed by the discoveries made in laboratories across the country, increased funding for cancer research from the federal government and other sources is essential if we are to continue to pursue a comprehensive understanding of the biology of cancer. First

and foremost, our ability to realize the exciting future that awaits us depends on a strong commitment by Congress to provide the necessary funding for the NIH and the NCI. As a country we must set priorities at this difficult time in our history—and the government can do no better with its money than continue to invest in medical research.

Second, we must cultivate a highly skilled and diverse workforce. The current research environment, hindered by a lack of funding, poses difficulties for all scientists. However, the challenges are particularly acute for young investigators. And it remains difficult to recruit and retain scientists from historically underrepresented groups.

Third, we must also continue to advance regulatory science so that as researchers develop innovative new approaches to conducting cancer research, the tools, standards, and techniques for assessing the safety and efficacy of all new products used to prevent, detect, diagnose and treat this disease continue to evolve. This is especially important in the realm of precision medicine, where research and development methods are continuously refined to incorporate advances in basic and clinical research.

Fourth, we must increase patient participation in clinical trials. Clinical trials are an essential component of cancer research and the only way to know if a therapy that appears promising in the lab will be safe and effective in a cancer patient. However, fewer than 5 percent of adult cancer patients participate in a clinical trial, and participation rates are even lower among patients from racial and ethnic minorities, among individuals over age 65, and people living in rural areas. If we are to speed the translation of cancer research discoveries to new treatments, it is essential that we do a better job of engaging patients in clinical research and removing barriers to participation in clinical trials. In recent years, lack of funding has been one of the most significant barriers researchers and patients have faced. For example, after the sequester cuts went into effect, approximately 750 fewer new patients were admitted to the NIH Clinical Center. Also, Phase I and Phase II clinical trials at cancer centers throughout the country were negatively impacted by the loss of funds.

The NIH needs stable, predictable increases in funding to continue to spur innovation and save lives Right now, the biological knowledge and the technological advances have positioned scientists at an inflection point. To pull back from federal investment is to abandon science in a time when scientists will be able to make quantum leaps in prevention and treatment of cancer. The AACR looks forward to working with you to ensure that researchers have the resources needed to continue to drive innovation and deliver hope to those who are confronting this dreaded disease.