Clifford A. Hudis, MD, FACP

President
American Society of Clinical Oncology

Testimony for the Record prepared for:
United States Senate Committee on Appropriations

Regarding Driving Innovation through Federal Investments

April 24, 2014

The American Society of Clinical Oncology (ASCO), the world's leading professional organization representing nearly 35,000 physicians and other professionals who treat people with cancer, appreciates this opportunity to provide insight on how federal investments in medical research drive innovation. This is a critical time for Congress to recognize the need for further investment to protect this valuable infrastructure for patients, the economy, and our nation's global competitiveness.

ASCO's members set the standard for cancer care world-wide and lead the way in carrying out translational and clinical research aimed at improving the screening, prevention, diagnosis and treatment of cancer. ASCO advocates for policies that provide access to high-quality care for all patients with cancer. ASCO's efforts are also directed toward supporting oncology clinical and translational research that is critical to improving the lives of our citizens and that can inform cancer services for people worldwide.

ASCO's *Clinical Cancer Advances* Report: The Latest Innovations

ASCO's *Clinical Cancer Advances 2013* report (http://www.cancerprogress.net/clinical-cancer-advances-2013) provides annual recognition of the major advances in patient treatments and care. The 2013 report details 76 research advances, 27 of which received NIH funding, in diseases impacting an estimated 1.6 million patients last year alone. Its top areas of progress include: using genomics to make treatment decisions for individual patients, discovering new cancer subtypes specifically associated with potential new therapies, tackling treatment resistant forms of cancer through precision medicine approaches, enhancing the ability of patients' own immune systems to fight cancer, and implementing new cancer screening paradigms to reduce disparities. Without the National Institutes of Health (NIH) and the National Cancer Institute (NCI), many of these questions would go unanswered and patients would be left without the best therapies.

As one concrete example, in 2013 we began to realize the the promise of targeted immunotherapy, one of oncology's most exciting new approaches. Tapping patients' own

immune systems can have a potent effect against a wide range of cancers, such as melanoma, kidney cancer, and aggressive acute lymphoblastic leukemia (ALL). What was recently just a novel idea was transformed into several pivotal advances that could soon change practice but this only happened because of federal investment in research.

Similarly, our efforts to decode the human, and tumor, genomes are now paying off. Recent studies shed new light on how genomics will eventually drive everyday patient care, helping doctors make better, more individualized treatment decisions as well as guide future drug discovery. The Cancer Genome Atlas network shared comprehensive molecular analyses of kidney and endometrial cancers as well as acute myeloid leukemia (AML). New molecular subtypes of endometrial cancer and glioblastoma were identified creating opportunities for advancement in treatment. The federally funded Lung Cancer Research Consortium reported success with testing patients for lung cancer driver mutations – then matching them with the best available targeted therapies and clinical trials. The genomic discoveries of recent years are being translated faster than ever into new, personalized therapies for actual patients – as well as into new leads for future drug discovery

NIH and NCI: Federal Investment Driving Medical Research Innovation

Federal funding in medical research is needed to study topics that the private sector typically doesn't pursue, such as comparisons of different but approved drugs, improving the quality and value of patient care, reducing cancer disparities, and developing cancer screening and prevention strategies.

At the same time, the NIH also takes on high risk, high reward research that cannot be done at its earliest stages by industry. This often groundbreaking work is many times picked up by the private sector and translated in to life-saving cures and economic growth.

An important consequence of NIH investment in research is that it is also a driving factor in many local economies. Fully 80 percent of NIH funding is distributed throughout the United States (US) and it is estimated that every dollar of NIH grant funding creates $2.21 of spending on jobs and businesses in our communities.

Clinical trials supported by federal funding have led to innovative breakthroughs in cancer care that touch every American family. Again, often these are in areas that industry has no incentive to pursue. Typically, the trial concepts are proposed directly by clinician investigators who hypothesize ways to improve treatments for their patients and want to test those hypotheses through rigorously designed prospective clinical trials. Just as the NIH RO1 and R21 grant

mechanisms inspire researcher creativity and innovation, the National Clinical Trials Network and National Community Oncology Research Programs are important in fostering research initiatives directly from clinician investigators who see firsthand the importance of answering questions vital to their patients.

Publicly funded clinical trials involve establishing comparative effectiveness, examining promising regimens, optimizing multimodality treatments, developing therapies for rare cancers, and studying prevention and survivorship strategies. These research goals may run parallel to those of commercial sponsors, but publicly funded trials are designed to benefit patients—not intended necessarily to achieve regulatory approval or shareholder interest. Many of these trials are at risk due to funding constraints and this can only slow the pace of further progress.

Food & Drug Administration (FDA): Carrying Innovation to Market

There are 12 million cancer survivors alive in the US today and this number is growing in no small part because of the new treatments FDA approves. ASCO is celebrating its fiftieth anniversary this year and with that we reflect on the great progress that has been made in cancer treatments over that time, much of which is due to the FDA's role in responsible drug approvals. The number of drugs available to treat cancer has grown from a small handful to more than 170, and options for toxicity management have vastly increased.

Despite the many achievements that ASCO celebrates this year, the Society and its members know we cannot afford to pause and trade on past success alone—there is too much at stake. An estimated 1.6 million Americans will be diagnosed with cancer this year, about 580,000 American lives were lost to cancer in 2013, and a growing, aging, and more overweight population makes it likely that cancer will replace heart disease as the leading cause of death by 2030 (with an increase of 40% in the total number of new cancer cases). Clearly, this is no time to slow the pipeline of development by underfunding the FDA.

Harnessing breakthroughs in technology and molecular biology will be the key to achieving better outcomes for patients. Under the new Breakthrough Therapies authority, 12 of the 28 FDA-designated Breakthrough Therapies in 2013 were targeted at cancer. We must keep accelerate this progress to make more rapid progress against cancer.

Current Threat to America's Global Leadership

While the US is slowing its investment in medical research, countries around the globe are making significant increases to theirs. Russia is increasing basic research funding by 65%, European investments are increasing by 40% over seven years, South Korea has pledged a 50% increase, and China announced a 26% boost in basic research funding in 2012. These investments result not only in additional research in these countries, but are attracting the best and brightest American-trained scientists to work abroad. The long-term consequences are

easy to predict. If scientific progress is achieved elsewhere, Americans will eventually find themselves being asked to import new treatments including drugs, intellectual property, and products.

Domestically, declining federal funding for clinical trials, coupled with the rising costs of increasingly complex studies, will severely harm the nation's clinical research enterprise by limiting opportunities for innovation and demoralizing young clinical investigators. As opportunities to develop and lead trials diminish and institutional pressures to generate research funding and clinical revenue continue to grow, young investigators may leave the field of research, or choose to pursue research opportunities in other countries. Not only does this threaten our progress against cancer, but it also diminishes the overall scientific workforce in America.

In addition, as clinical trials are increasingly conducted overseas due to the costs and regulatory complexities of conducting them in the US, our own patients, and your constituents, may be denied the opportunity to participate as treatments are developed. They may be delayed in receiving the most promising potential treatments and physicians or research nurses are left out of exciting areas of research. Congress should demonstrate a continued commitment to ensure biomedical research is federally funded for the benefit of American patients and American scientific leadership.

Without maintenance of those investments, our global leadership and the benefits it offers everyday Americans in both health and economically, is profoundly threatened.

Need to Renew Investment

Because of the incredible scientific opportunities facing us and the current threats to this opportunity, ASCO urges the Committee to provide the necessary investments to the NIH, NCI, and FDA in fiscal year (FY 2015) to protect innovation. Specially, we offer the following recommendations for FY 2015 funding:

- National Institutes of Health (NIH): $32 billion
- National Cancer Institute (NCI): $5.26 billion
- Food & Drug Administration (FDA): $2.784 billion

ASCO again thanks the Committee for its continued support of cancer patients in the US through funding for the NIH, NCI, and FDA. We look forward to working with all members of the Committee to advance US cancer research.

Questions? Contact Amanda Schwartz, ASCO Government Relations Specialist, Amanda.schwartz@asco.org or 571-483-1647.