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June 1, 2014

Chairman Fred Upton
Chair, Energy and Commerce Committee
2125 Rayburn House Office Building
Washington, DC 20515

Representative Dianna DeGette
2368 Rayburn House Office Building
Washington, DC 20515

Dear Chairman Upton and Representative DeGette,

On behalf of the nearly 35,000 members of the American Society of Clinical Oncology (ASCO) who specialize in the treatment of patients with cancer, I commend you for launching the 21st Century Cures Initiative to examine how to accelerate the discovery, development, and delivery of promising new treatments to patients. I am pleased to provide input on the first white paper, 21st Century Cures: A Call to Action.

As ASCO celebrates its 50th anniversary this year, we reflect on the tremendous progress made in cancer treatments over that time. The number of drugs available to treat cancer has grown from a small handful to more than 170, many of which are far more effective and less toxic than previously available treatments. Today more than two-thirds of patients with cancer are alive five years after their diagnosis, compared with less than one-half in the 1960s. There are now more than 13 million cancer survivors alive in the United States (US) and this number is growing.

Despite these achievements, there remain many unmet medical needs for cancer patients, and this is not the time to slow development. This year, an estimated 1.6 million Americans will be diagnosed with cancer. In 2013, about 580,000 American lives were lost to cancer. The population is growing, aging, and more overweight, making it likely that cancer will take over heart disease as the leading cause of death by 2030.

ASCO appreciates the work of the Office of Hematology and Oncology Products (OHOP) led by Dr. Richard Pazdur at the Food and Drug Administration (FDA). OHOP oversees development, approval, and regulation of drug treatments for cancer, therapeutic biologic treatments for cancer, therapies for prevention of cancer, and products for treatment of nonmalignant hematologic conditions.

Scientists within OHOP are working on incorporating innovations in pharmacogenomics, bioinformatics, and clinical trial design into the drug review process. ASCO has cosponsored workshops with OHOP and others to better measure

2318 Mill Road, Suite 800
Alexandria, VA 22314
T: 571-483-1300
F: 571-366-9530
www.asco.org

the status of disease in patients with leukemia, define surrogate endpoints for neoadjuvant breast cancer trials, educate scientists and advocates about anticancer agent development, and convene stakeholders to create a genomic-based master protocol for metastatic breast cancer trials. OHOP has been a leader within FDA in using approval pathways such as Accelerated Approval to get drugs to patients faster, and has quickly adopted new programs such as the Breakthrough Therapies Designation. These efforts have contributed substantially to accelerating the introduction of new treatments for cancer into practice. ASCO is concerned, however, about the ability of the FDA in general and the OHOP specifically to continue to expand the scope and quality of their work without additional resources.

ASCO's 2011 report, *Accelerating Progress Against Cancer: ASCO's Blueprint for Transforming Clinical and Translational Cancer Research*, presented a vision for cancer research and patient care to become more targeted, efficient and effective. The Blueprint, attached, contains recommendations that address many of the issues the 21st Century Cures Initiative is exploring:

1. Establish a new approach to therapeutic development, driven by our more thorough understanding of cancer biology and the advent of new technologies.
2. Design smarter, faster clinical trials to provide evidence for effective treatments targeted to patients most likely to benefit.
3. Harness advances in health information technology to seamlessly integrate clinical research and patient care.

Subsequently, ASCO released *Shaping the Future of Oncology: Envisioning Cancer Care in 2030*, which presents a long-term vision of cancer care and outlines potential barriers. This report identifies three key drivers of change that are likely to have the biggest impact on cancer care over the coming decades:

1. "Big data." Rapid advances in health information technology (HIT) have created unprecedented opportunities to collect, analyze and learn from vast amounts of real-world data.
2. Cancer panomics. We are coming to understand the complex networks of molecular pathways and characteristics of the tumor microenvironment that interact to drive cancer and will need to be targeted, in combination, to develop prevention strategies and curative therapies.
3. Delivering value. Unsustainable cost increases and improvements in quality metrics are leading to a growing focus on cost effectiveness and "value" in health care.

Since issuing these reports, ASCO has worked with partners to drive the report's recommendations forward. Many other major stakeholders, including the National Cancer Institute (NCI) and the FDA, have also launched initiatives that will contribute to achieving the vision. Together, these steps represent significant new momentum toward a research system that realizes the potential of precision medicine.

We must reinforce these efforts by reexamining the traditional processes and assumptions in the development and delivery system, especially as we transition to an era where molecularly targeted agents will become increasingly more common. Inefficiencies in the clinical trial process exist and must be resolved at many levels: among the research community, providers, payers, at the National Institutes of Health (NIH) and the FDA. As a community, we need to rethink the way that safety is assessed in trials

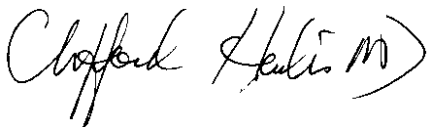
of molecularly targeted treatments. To address this and other issues, ASCO is developing recommendations on modernizing eligibility criteria for clinical trials. With respect to pediatric cancer research, trials of promising new agents need to begin sooner and incentives need to be realigned. Currently, most pediatric cancer trials start only after completion of the adult pivotal trial. In order to speed drugs to children, trials should begin while the adult trials are ongoing. In addition, the pediatric patent extension program needs to be revisited. Currently, the program extends the patent of a drug if it can be used for the same disease in children. Children often do not suffer from the same diseases as adults, but we are discovering that some of the molecular targets of adult and pediatric cancers are the same. The patent extension should apply to drugs that can treat effectively pediatric cancers, even if it is not the same cancer as the adult indication.

We support the development of truly superior treatments. In March 2014, ASCO released a perspective, *Raising the Bar for Clinical Trials by Defining Clinically Meaningful Outcomes*, which calls on the community of patients, patient advocates, and clinical investigators to collectively raise the bar in our expectations of the benefits of new therapies and to design clinical trials to demonstrate greater benefits. It outlines goals for cancer clinical trials in several diseases that researchers should aim for, and patients should expect. The recommendations provide examples of "clinically meaningful outcomes" for advanced pancreatic, lung, breast, and colon cancers.

To accelerate progress, we suggest that you hold a roundtable discussion focused on oncology. Given the nature of the disease and the nation's longstanding investment in cancer research, the field of oncology has already dealt with many of the issues that have now begun to arise in other disease areas. An examination of what has worked, and what hasn't, would likely benefit your initiative.

Thank you for the opportunity to provide input. We look forward to working with you on the 21st Century Cures Initiative and offer ASCO as a resource to you.

Sincerely,



Clifford A. Hudis, MD, FACP
President
American Society of Clinical Oncology