NCCS
NATIONAL COALITION FOR CANCER SURVIVORSHIP
The power of survivorship. The promise of quality care.

June 13, 2014

The Honorable Fred Upton
Chairman
Energy & Commerce Committee
House of Representatives
Washington, D.C. 20515

The Honorable Henry Waxman
Ranking Member
Energy & Commerce Committee
House of Representatives
Washington, D.C. 20515

The Honorable Diana DeGette
Energy & Commerce Committee
House of Representatives
Washington, D.C. 20515

Dear Chairman Upton, Representative Waxman, and Representative DeGette:

The National Coalition for Cancer Survivorship (NCCS) represents survivors of all forms of cancer in public policy advocacy aimed at improving the quality of cancer care. We appreciate the opportunity to comment on the 21st Century Cures initiative undertaken by the Energy & Commerce Committee. NCCS is dedicated to providing every cancer patient the tools and opportunity to make informed decisions about his or her care and to removing obstacles to delivery of high-quality, evidence-based cancer care.

The 21st Century Cures effort provides an important opportunity to reflect on significant recent advances in cancer treatment and to assess barriers to development of new cancer treatments and failures in providing care for those who live with cancer as a chronic disease. In our comments below, we address the Call to Action and the questions that were posed to patient organizations as part of the 21st Century Cures fact-finding effort.

NCCS does not directly fund cancer research but instead focuses its efforts on creating the optimal environment for research, development, and approval of new cancer therapies and ensuring patient access to quality care.

Developing Cures While Enhancing Care

We applaud the ambitious goals of the 21st Century Cures initiative. As the committee seeks advice about creating a research and development climate that fosters bold treatment advances and cures, we urge that the needs of those who are living with and managing cancer as a chronic disease not be forgotten.

Research and development advances of the last several decades have turned cancer into a chronic disease for many, a welcome and important accomplishment. Cancer is in fact many different diseases, and most of them have humbled researchers because of their complexity and ability to adapt in the face of new treatments.

Pursuing cancer cures is an important aspiration. However, this pursuit should not be at odds with the development of cancer therapies that are transforming some forms of cancer into manageable chronic diseases.

The complexity of cancer and the difficulty of curing all cancers have implications for your effort and for the way in which the National Institutes of Health (NIH) and Food and Drug Administration (FDA) approach their cancer related responsibilities. While NIH investments should be made in ambitious research and development to cure cancer, there must also be an emphasis on developing treatments that are accompanied by fewer side effects and that help cancer survivors maintain a high quality of life with cancer as a chronic condition. In addition, greater investments should be made in understanding the late and long-term effects of cancer treatment. Those who might be "cured" of cancer are often at high risk of long-term side effects of treatment, including second cancers. We urge that the 21st Century Cures effort take a comprehensive and long-term approach to the concept of cancer treatment and cure.

Reconsidering the concept of treatment and cure will also have implications for FDA. Although we agree that FDA reviewers must be prepared for the development and review of targeted and personalized therapies (as we discuss below), so should they be prepared for evaluation of drugs that permit chronic management of cancer and that may be accompanied by significant late and long-term effects.

Achievements of the Office of Hematology and Oncology Office within the Center for Drug Evaluation and Research

The "Call to Action" document asks a number of important questions about the role of FDA in therapeutic development. For example, the white paper asks, "Is FDA structured and managed to enable the agency to rapidly incorporate innovative new approaches and technologies into its review processes? How can Congress ensure that the regulatory science keeps pace with advances in personalized medicine, including diagnostics?"

We direct the committee's attention to the record of the Office of Hematology and Oncology Products in the Center for Drug Evaluation and Research, as we think the office's work might serve as a model for other review offices. The achievements of the office are many: 1) approval of a number of new agents according to accelerated approval standards, followed by careful monitoring to assure completion of post-approval confirmatory trials; 2) utilization of the breakthrough therapy designation authorized by the Food and Drug Administration Safety and Innovation Act (FDASIA) as a means of creating an open and less bureaucratic review process; 3) communication with researchers, patients, and sponsors to evaluate surrogate endpoints that might support accelerated approval; 4) publication of peer-reviewed articles and agency guidance documents outlining new pathways for development and review of cancer treatments, including a guidance on pathologic complete response as an endpoint for neoadjuvant breast cancer treatments, 5) aggressive management of adverse effects of effective cancer treatments, to ensure the continued use of products when warranted, and 6) an culture of open communication and collaboration with patients, researchers, and sponsors.

The achievements of the Office of Hematology and Oncology Products could be replicated in other therapeutic areas, but not without direct action to foster such efforts. A conscious effort to foster efforts parallel and complementary to those of the Office of Hematology and Oncology Products would also provide benefits to the office itself. More flexibility in hiring at FDA is warranted. For example, approaches that would permit hiring of clinicians and clinical researchers who split their time between NIH and FDA would help to ensure that FDA has the scientific and clinical experience it needs in an age of targeted therapies. In addition, those who are on staff at FDA should be permitted to maintain the professional and scientific relationships that are critical to their professional development and to their ability to review cutting edge products. Fostering a climate for intellectual and professional development may require a reconsideration of conflict of interest policies and also the appropriation of funds to permit travel and attendance at professional meetings. These are the settings in

which cutting edge science is discussed, but it is increasingly difficult for FDA officials to attend, as they may be thwarted by lack of travel funds or unnecessarily restrictive conflict of interest policies.

Many of the answers to improving the performance of FDA exist within FDA. We encourage the committee to evaluate and replicate successful performance at the agency.

Rewarding Innovation while Ensuring Access to New Therapies

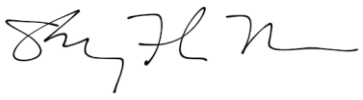
In its materials defining the 21st Century Cures effort, the committee poses questions about the adequacy of rewards for innovation by biotechnology and pharmaceutical sponsors. These are difficult questions, and to date American society has been reluctant to see the health care system or the political system set standards for appropriate rewards for innovation. We have instead preferred to see the rewards for innovation set by market forces.

Health care consumers are increasingly confronting difficulties obtaining access to life-saving therapies. The obstacles are many: consumers may not be able to afford the cost-sharing responsibilities that accompany expensive but potentially life-saving new therapies or patients' insurance companies may impose coverage standards that block a patient's access to a new therapy. In the background is the question of whether the prices of new therapies necessarily reflect their benefits.

A movement is developing to strengthen the communication to patients about the cost of their treatment. As an organization that supports frank communication about cancer care options, we would include communication of cost of care in that effort. However, the patient who is making a treatment decision is not in a position to make determinations about the value or overall cost of his or her care. Patients should not be put in the position of making political judgments about the value of the treatments they are considering. We urge a broader consideration of this issue outside the patient-doctor communication. We specifically urge the committee to accompany any consideration of appropriate reward to innovators with consideration of the manageable cost of care for consumers, including their cost-sharing responsibilities. Cures will not be realized if consumers cannot afford them.

We appreciate the opportunity to comment on the 21st Century Cures initiative. We will continue to monitor the work of the committee and comment on additional questions and issues you pose for public comment.

Sincerely,



Shelley Fuld Nasso
Chief Executive Officer

cc: The Honorable Joe Pitts
The Honorable Frank Pallone