

CANCER LEADERSHIP COUNCIL

A PATIENT-CENTERED FORUM OF NATIONAL ADVOCACY ORGANIZATIONS
ADDRESSING PUBLIC POLICY ISSUES IN CANCER

March 27, 2015

The Honorable Fred Upton
Chairman, Committee on Energy & Commerce
United States House of Representatives
Washington, DC 20515

The Honorable Diana DeGette
United States House of Representatives
Washington, DC 20515

Dear Chairman Upton and Representative DeGette:

The undersigned organizations represent cancer patients, physicians, pharmacists, researchers, and other health professionals who are engaged in efforts to improve cancer treatment and enhance the overall quality of cancer care. We appreciate the opportunity to comment on the January 2015 discussion draft, "21st Century Cures Act."

Our comments will focus on the following objectives:

- Balancing the speed of regulatory review against an assurance that new cancer drugs are safe and effective;
- Preparing for review of precision medicine drugs;
- Ensuring that "patient-focused drug development efforts" are reflected in FDA programs and regulatory approaches;
- Encouraging the consideration of patient-reported outcomes data in the review process;
- Building data-collection and sharing efforts on a firm foundation of successful clinical trials data reporting;
- Defining the new roles of patient advocacy and patient research foundations in the therapeutic development process; and
- Ensuring that new commissions, panels, and reports serve the needs of patients, do not duplicate existing commissions and reporting requirements, and do not create unreasonable burdens for federal agencies.

Ensuring a Strong Regulatory Review Process

Cancer patients, physicians, and other health care providers have an interest in eliminating any inefficiencies in the regulatory review process and ensuring patients access to safe and effective drugs at the earliest possible time. However, we want to be sure that those drugs that are approved by the Food and Drug Administration (FDA) are safe and effective and will provide clinical benefit to patients.

Cancer patients and their health care teams have benefited greatly from the efforts of the Office of Hematology and Oncology Products to improve the cancer drug review process and expedite the review of cancer drugs whenever possible. The Office, within the Center for Drug Evaluation and Research (CDER), has made aggressive but appropriate use of the expedited programs for serious conditions, as defined by the Guidance for Industry dated May 2014. These expedited programs include fast track designation, breakthrough therapy designation, accelerated approval, and priority review designation.

The 2014 review record of the Office of Hematology and Oncology Products is impressive. Drugs were approved for treatment of advanced ovarian cancer, acute lymphoblastic leukemia, three types of blood cancer, metastatic non-small cell lung cancer, and melanoma. This drug review and approval record was accomplished through use of the expedited development and review pathways; Prescription Drug User Fee Act (PDUFA) goals were met in almost all 2014 drug reviews and most drugs were approved on the first review cycle.

In light of the record of Office of Hematology and Oncology Products, we offer cautions about two proposals that are included in the draft. First, we are not persuaded that confirmatory trial requirements should be eliminated for those drugs that are subject to accelerated approval. Those requirements should remain in place for those drugs approved on the basis of surrogate endpoints. Second, we are concerned about a suggestion that supplemental approvals might be based on summaries of data, without a requirement of submissions of the underlying data. FDA has a proven track record for efficient review of cancer drugs, and changing the amount of data necessary for an application is neither necessary nor advisable.

Instead of eliminating post-approval study requirements or changing data requirements for approval, we encourage evaluation and replication of the work of the Office of Hematology and Oncology Products. That effort will identify effective ways to utilize current expedited review mechanisms.

Preparing for Review of Precision Medicine Drugs

Although we are pleased with the performance of the cancer review office to date and applaud the willingness of the office staff to collaborate with patient advocacy groups and professional societies on issues ranging from clinical trial design to identification of surrogate endpoints, we see significant challenges for the office and for all of FDA in the future.

As we move more completely into the age of precision medicine, the office will need assurance that all personnel possess the skills for review of targeted therapies. In addition, FDA reviewers need more flexibility to attend and participate in scientific and medical meetings. These meetings are an opportunity for continuing medical education and for staying current on developments related to precision medicines, and these opportunities should be available to review staff.

We note that the committee has left in its discussion draft a “placeholder” for FDA personnel issues. We urge that this placeholder be replaced by revisions to FDA authority that will streamline hiring processes. In addition, travel and ethics rules should be addressed – if necessary, in legislative language – to guarantee FDA staff the ability to attend important meetings in their field.

Patient-Focused Drug Development Efforts

The Food and Drug Administration Safety and Innovation Act (FDASIA) included a number of important patient-focused drug development efforts. The patient-focused drug development meetings have been of special interest to patient advocates. We appreciate that the agency recognizes the importance of involving patients in drug development issues consistent with FDASIA requirements. Although we are concerned about adding responsibilities to the portfolios of review teams, which should be primarily focused on new product review, we would like to see more engagement of reviewers in the planning and execution of the patient-focused drug development meetings. This is the most efficient means of ensuring that the patient-focused meetings undertaken by the agency are integrated into the operations and inform the thinking of the agency.

Patient-Reported Outcomes in the Regulatory Review Process

The initial section of the discussion draft encourages the use of patient experience data to inform the risk-benefit assessment. We are pleased that the draft seems to encourage serious consideration of patient-reported outcomes in the regulatory process, but we recommend more specific definitions be included in this section of the bill. If patient-reported outcome data are to be utilized in a data-driven regulatory process, the standards for those data must be well-defined. It will not benefit patients if the agency is encouraged to consider patient anecdotes that do not meet reasonable data standards.

The committee should consider setting goals for approval of patient-reported outcome tools by the agency and encouraging reference by the agency to the information provided through those validated tools.

Building Successful Data-Collection and Data-Sharing Initiatives

We are strong supporters of a movement toward “big data” collection and sharing to fuel strong cancer drug development and clinical care improvement. In fact, a number of our organizations have developed data registries that track the treatment and outcomes of our patients. We urge that any federal involvement in data collection and sharing efforts be built on a strong foundation. To that end, we encourage that recent findings of limited compliance with the

reporting requirements of www.clinicaltrials.gov be considered by the committee. These findings should inform efforts to strengthen clinical trials reporting. In addition, a stronger trials results reporting system might serve as a foundation for other data collection efforts.

Defining the Roles and Responsibilities of New Commissions and Panels

A review of the discussion draft raises some concerns related to the number of new commissions, consortia, and reporting requirements that are authorized. Our reservations are two. First, we are concerned that some of the new research and regulatory efforts and initiatives may be redundant of existing research and regulatory programs. For example, has the National Center for Advancing Translational Sciences been evaluated to determine if parallel clinical research programs are necessary? Has the regulatory science collaboration between the National Institutes of Health (NIH) and FDA been reviewed? What are the results of the Critical Path Initiative?

Second, we are concerned that the new consortia, commissions, and reports will be accompanied by significant costs that cannot easily be absorbed by NIH and FDA and that additional resources for these responsibilities will not be available.

Understanding the Roles of Nonprofit Research Foundations

If the 21st Century Cures Consortium and the Medical Products Innovation Advisory Commission are retained after the committee considers any possible overlap with existing programs and the cost associated with new commissions, we recommend that membership of both groups be redefined to include more members drawn from patient advocacy organizations and robust representation from non-profit, patient-driven research foundations. The Cures Consortium would number 22 members, including 8 representatives of the biopharmaceutical and medical device industries and 9 who shall be “representatives of academic researchers, patients, health care providers, and health care plans and insurers, to be appointed by the Comptroller General of the United States, after soliciting nominations.” The Medical Products Innovation Advisory Commission would include 17 members, and the discussion draft does not indicate that any will be patient advocates or representatives of non-profit research foundations.

We believe that the membership categories for both of these panels should be redrafted to ensure strong representation of patient advocates and inclusion of individuals from non-profit research foundations. Patients can speak to unmet medical needs, and those representing research foundations may also bring extensive experience and expertise about research and development of new treatments. Over the last decade, there has been nothing short of a revolution in the operation of patient-driven research foundations. These groups have refined the manner in which they invest their resources, expanding beyond investigator-initiated grants to therapy development programs. In addition, many of them have been innovators in clinical trial design and recruitment and are pioneering data collection and sharing efforts. Their expertise must be reflected in the deliberations of these commissions, and that can be accomplished by guaranteeing robust membership from their ranks.

Ensuring Access to New Therapies

We note that the discussion draft focuses primarily on the development and regulatory review of new therapies, and we have confined our comments to those topics. However, it is critical that cancer care delivery systems ensure patients access to the treatments of the 21st century. We are actively involved in payment and delivery reform efforts that will ensure access to quality, affordable, and sustainable cancer care.

Thank you for the opportunity to participate in the process of developing legislation to encourage development of new treatments for the new century.

Sincerely,

Cancer Leadership Council

Association for Molecular Pathology
CancerCare
Cancer Support Community
Fight Colorectal Cancer
Hematology/Oncology Pharmacy Association
International Myeloma Foundation
Kidney Cancer Association
The Leukemia & Lymphoma Society
LIVESTRONG Foundation
Lymphoma Research Foundation
Multiple Myeloma Research Foundation
National Coalition for Cancer Survivorship
National Patient Advocate Foundation
Ovarian Cancer National Alliance
Pancreatic Cancer Action Network
Prevent Cancer Foundation
Sarcoma Foundation of America
Us TOO International Prostate Cancer Education and Support Network