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# Statement of Leonard Lichtenfeld, M.D.
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## "21st Century Cures: Incorporating the Patient Perspective"

## United States House of Representatives
## Committee on Energy and Commerce
## Subcommittee on Health

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Chairman Pitts, Ranking Member Pallone and Members of the Subcommittee:

I am Dr. Leonard Lichtenfeld, Deputy Chief Medical Officer for the American Cancer Society. On behalf of the Society, thank you for the opportunity to testify today. The Society is a nationwide, community-based voluntary health organization dedicated to eliminating cancer as a major health problem by preventing the disease, saving lives, and diminishing suffering through research, education, advocacy, and service. The Society, operating through its national office and 12 geographic divisions throughout the United States, is the largest voluntary health organization in the United States.

We are pleased to have the opportunity to contribute to the Committee's 21st Century Cures initiative – in particular to illustrate the importance of increasing and promoting the participation of patients in drug and device research and approval. No one knows better what is at stake in the quest for new and better treatments for cancer than cancer patients.

Patients must be the focus of innovation – and they must also be active partners in all aspects of research, development and regulation of new therapies.

Today, there are nearly 14 million cancer survivors in the United States thanks to more effective treatments and improved screening tools made possible through research. This research is funded every year by institutions like the National Institutes of Health (NIH) and the National Cancer Institute (NCI); by not-for-profit organizations like the American Cancer Society, other foundations, and universities; and privately by pharmaceutical companies. All of this research opens the door, and provides a pathway to patients, for new FDA-approved medications, therapies and devices that greatly impact patient quality of life. We must continue and expand our steadfast commitment to research – and we must continue to support researchers who are working on

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finding the next generation of cures. Just as importantly we must renew our commitment to making sure that the investments in research that translate into drugs and devices that are expedited through the approval process are appropriately *safe*, *effective*, and *accessible* to patients.

The Food and Drug Administration Safety and Innovation Act (FDASIA) that was signed into law in 2012 addressed the need to include patients to a greater extent throughout the FDA drug and device approval process. ACS CAN championed the inclusion of Sections 1137 and 1142 in FDASIA to expand the FDA's Patient Representative Program and its mission to maximize patient input during the drug development process. In addition, FDASIA also required FDA to hold a series of patient-focused drug development meetings on various diseases.

FDA has included patient participants in important advisory committee meetings since the early 1990s. But the FDASIA provisions codified the requirement for patients to be involved in the process of drug development and review, and we must continue to build on this progress. Patient representatives routinely serve on public FDA advisory committees that review products and therapies for the diagnosis and treatment of serious diseases such as cancer, and also serve as consultants to the FDA review division, participating in FDA/sponsor meetings where they may contribute to clinical trial design discussions, and labeling negotiations. When patients participate in these important meetings they can provide FDA and industry with important patient perspectives at various stages of medical product development and regulation, including pre-clinical, clinical trial design, and endpoints. They can share what is most important to patients – such as symptom reduction, risk tolerance, and design elements that might affect trial recruitment or retention.

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All patients, and specifically cancer patients, can provide a unique perspective on the benefits and risks of particular therapies being considered for FDA approval. This kind of patient involvement should be reinforced and supported. Provisions included in FDASIA requiring offices inside FDA to collaborate and address challenges that have historically hindered patients from participating in these important discussions must be fully implemented.

The Committee should also consider examining opportunities for providing greater funding to support the FDA Patient Representative Program, as well as broader continued engagement with the patient community. The series of patient-focused drug development meetings required in FDASIA that have been held to date have yielded successful conversations between FDA and patient groups. However, there is a need for continued communication and planned actions in the disease areas FDA identified.

One of the important ways that patients' perspectives can inform the development of therapies is through the design and the use of patient-reported outcomes (PROs) in research. While it may seem intuitive that the patient's outcome with a new treatment is the most important outcome, the current research and drug approval process focuses heavily on biologic or physician-reported outcomes. Common measures of the effectiveness of cancer therapies include overall survival, progression-free survival, time to progression, or tumor shrinkage. Sometimes functional status, pain or quality of life measures are included, but they may be reported by the physician rather than by the patient. Research has shown that when comparing a physician's measure of a patient's pain with the patient's own perception, the two measures can differ significantly. Many of the quality of life measures like pain, nausea, fatigue, depression or ability to carry out normal daily activities should come from patients themselves. Not only can patients provide important feedback

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on these symptoms, but they can also help prioritize the importance of these side effects in the overall response to a disease and the associated treatments. When these quality of life outcomes are rigorously measured and supported by the FDA, they can and should be included in a drug's labeling, and can, by themselves, be a basis for a drug's approval. The FDA should be encouraged to work with industry and researchers to incorporate self-reported symptom measurements as a regular part of clinical trials, including, for example, new technologies for monitoring patient's experiences with new treatments.

The goal of the 21st Century Cures Initiative is to accelerate the development and approval of new medical treatments. There are few other areas that can match the research and development activity seen in the field of cancer, and while there remains an incredible amount of progress to be made before we have safe and effective treatments for the hundreds of diseases we call cancer, cancer drug development has served in many ways as a model of innovation. In past years Congress has provided FDA with a number of tools to accelerate and simplify the approval process for drugs. FDA's Office of Hematology and Oncology Products (OHOP) has been aggressive about using the full complement of these tools to speed new drugs to patients and has encouraged drug companies to be innovative in their clinical trials. In the past eight months, three cancer drugs have been approved using the accelerated pathway. One approval was based on a trial of only 111 patients, another with only 163 patients and one with 129 patients. These are examples of the types of research and approvals that are happening faster, and with smaller clinical trials than in the past. Small-sized trials and accelerated approval, however, have drawbacks. They may not be able to include a diverse population in terms of race, age, sex and comorbidities, which may provide an incomplete picture of how a drug might work in a broader application, and later data may change

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our understanding of the risk-benefit ratio. These small trials and accelerated approvals also tend to be seen in cancers that are deadly and have no other good therapeutic options. I want to stress that the risk-benefit tolerance of a cancer patient facing a poor prognosis may be very different than the risk tolerance of a patient with a less deadly or more chronic disease with other available treatment options. Even within the field of oncology, it is important to note that the long-term effects of some treatments have different implications for children with cancer as opposed to older adults. Research and drug approval in the field of oncology might be seen as leading other fields, and FDA's flexibility should be continued, but the same acceptance of reduced data on which to base FDA approval on might not be appropriate in other fields or for other diseases.

In closing, I would like to reiterate the vital role of patients in the research, development, and drug approval process. Cancer patients have the most at stake in the quest to find new treatments and have important insight to offer. Every disease is unique in terms of the unmet need for therapies and the risks that patients are willing to accept in the quest for new treatments. If researchers, pharmaceutical companies and the FDA engage widely and meaningfully with patients, the result will be better treatments delivered to patients faster.

Thank you again for the opportunity to share our views.