LEUKEMIA & LYMPHOMA SOCIETY®
fighting blood cancers

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Division of Dockets Management
US Food and Drug Administration
5630 Fishers Lane
Rockville, MD 20852

RE: Docket No. FDA-2014-N-1968, Food and Drug Administration Activities for Patient Participation in Medical Products Discussions

Dear Assistant Commissioner Kux:

The Leukemia & Lymphoma Society (LLS) appreciates this opportunity to submit comments on the Food and Drug Administration's (FDA) activities for patient participation in medical product discussions. As the world's largest voluntary health agency dedicated to the needs of blood cancer patients, LLS is very pleased with and supportive of the agency's ongoing efforts to ensure that the patient voice is heard in the development of medical products and during regulatory discussions.

Each year, over 140,000 Americans are newly diagnosed with blood cancers, accounting for nearly 10 percent of all newly diagnosed cancers in the United States. The mission of LLS is to find cures for leukemia, lymphoma, and multiple myeloma and to ensure that blood cancer patients have sustainable access to quality, affordable, coordinated healthcare. Since our founding 65 years ago, LLS has invested over $1 billon into research for cures and LLS-funded research has been part of nearly all of the FDA-approved therapies for blood cancer treatment.

As the FDA looks for ways to improve patient participation in medical products discussions, LLS strongly recommends the following:

- Integrate the patients' voice early and often.
- Continue to strengthen relationships with patients groups.
- Conduct a specific blood cancer Patient-Focused Drug Development Initiative meeting
- Use process consultation meetings to improve the Patient-Focused Drug Development Initiative
- Develop a blueprint for patient organization-sponsored patient-focused drug development meetings.
- Develop templates to facilitate the use of Patient Reported Outcomes data.
- Examine and eliminate barriers to appropriate patient-sponsor interactions.

LLS appreciates the various initiatives the FDA has launched to date to capture the patient perspective in medical product development discussions and regulations to ensure that the products that are brought to market are ones that patients both want and need. LLS has participated in these efforts through patient representative program. The patients LLS has nominated are pleased to be part of the development process and we are grateful that the FDA recognizes the wealth of information and personal experience these patient bring to the table. We encourage regular interaction with these patients so that the patient experience is used both to inform development decisions and formulate the questions that are asked.

LLS also appreciates the FDA's willingness to meet with LLS and other patient groups on issues of importance to the patient community. These meetings serve to assist patient organizations in developing research and policy agendas that compliment the work being done by the FDA, the National Institutes of Health (NIH) and industry. Because of our experience in medical research, patient support services, and health policy, LLS is uniquely qualified to provide the agency with feedback on the real world needs of patients with blood cancers, including the impact of disease on patients' lives, the measures of benefit that matter most to patients, and the effectiveness of existing treatments.

LLS is also pleased with the FDA's Patient-Focused Drug Development Initiative. However, we have some concerns around the lack of transparency in the selection of the diseases to be considered in future meetings. While LLS agrees with the concept of applying specific criteria to the selection process, it is unclear how these same criteria were applied in selecting the diseases that were included in the final list. To address this issue, we propose that the FDA consider reengaging patient groups in process consultations meetings, similar to those held during the development stages of this initiative in 2012 and 2013. These consultation meetings will bring further clarity and transparency to the final disease selections and take advantage of the lessons learned from public meetings held to date.

Finally, similar to the last time that such a list was developed, again no blood cancers were included in the recent list of diseases proposed for public meetings. We have submitted, under separate cover, our recommendations to include Myelodysplastic Syndromes (MDS) and Chronic Lymphocytic Leukemia (CLL), particularly egregious diseases, to the list of diseases to be addressed in fiscal years 2016-2107. We sincerely hope that you will correct this omission by adding the aforementioned diseases.

Additionally, if the FDA intends to continue this initiative beyond the CY years 2016-2017, the agency might consider focusing on broader disease areas, such as blood cancers, neurodegenerative disease, cardiovascular disease, or autoimmune disease as this would eliminate the almost impossible task of prioritizing 7,000 distinct diseases and would ensure that a broad base of patient stakeholder voices are taken into consideration.

LLS appreciates that the FDA is also willing to work concurrently with individual patient groups who wish to host their own FDA-patient meetings. We recommend that the agency publish a roadmap or blueprint for developing such meetings, e.g. FDA staff or department contacts, data collection recommendations and sharing of meeting findings with the FDA.

LLS supports the FDA's efforts under the Patient Preference Initiative to provide information, guidance and a framework necessary to incorporate patient preferences on the benefit-risk tradeoffs of medical devices. Recent advances in precision medicine are beginning to make it possible to provide patients with precision-based treatments and an integral component to this precision-based approach are diagnostic tools. LLS supports efforts to understand patient preferences on the benefit-risk tradeoffs for these medical devices.

As the FDA is aware, Congress recently launched the 21st Century Cures Initiative to take a comprehensive look at steps to accelerate the pace of cures and medical breakthroughs in America. LLS is actively engaged in this initiative and has submitted comments to the House Energy & Commerce (E&C) Committee around patient reported outcomes (PROs). PROs are essential tools that allow regulators, clinicians, and patients to better understand the clinical and practical impact of a therapy.

PROs are structured, self-reported information provided directly by the patient regarding his or her condition or experience, and which are by definition received without interpretation by the health care provider or other entity.

Our comments to the E&C Committee recommend that the FDA be empowered to streamline the incorporation of PROs data into clinical trials. The availability of PRO information could significantly alter the benefit-risk analysis conducted by the FDA during the review process. Furthermore, PRO data is critical to enable patients and doctors to fully weigh the advantages and disadvantages of a particular intervention and make a fully informed decision on treatment options. LLS acknowledges the FDA's efforts in regards to PROs, including the 2009 Final Guidance on the use of PROs to support drug approvals and labeling changes. Despite this Guidance and extensive work by the PRO Consortium and other stakeholders, sponsors often forgo PRO measures during clinical trials in order to avoid the potential time lost to an unpredictable PRO design and validation process. From a patient perspective, this is a missed opportunity and one that we urge the agency to address in the near future.

LLS recommends that the FDA establish a transparent process to work with industry and the patient community to develop template frameworks for PROs to streamline the inclusion of substantive, useful PRO data within clinical trials intended to support an approval or new labeling claim. These templates should include best practices for survey content and systematic data collection methods which are tailored for specific disease areas and other variations.

LLS is also concerned by the current maze of existing and perceived legal barriers preventing drug sponsors from engaging directly with patients early in the development and trial design process. While some barriers provide legitimate protection to vulnerable consumers, others are antiquated and do not meet the needs of the modern drug development process. LLS has recommended to the E&C Committee that a thorough review delineating the legal barriers to manufacturer engagement of patients be conducted. The results of such a review could be used to enhance the drug development process and remove barriers to patient-sponsor engagement.

Thank you for the opportunity to submit these comments. We trust that the FDA will find these suggestions helpful to the agency' efforts to incorporate the patient voice in medical product discussions and regulations.

Sincerely,

Brian Rosen
Chief Policy, Advocacy & Patient Access Officer
The Leukemia & Lymphoma Society