LEUKEMIA & LYMPHOMA SOCIETY®
fighting blood cancers

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April 13, 2015

Division of Dockets Management
US Food and Drug Administration
5630 Fishers Lane
Room 1061
Rockville, MD 20852

RE: Docket No. FDA-2015-D-0268-0001
Individual Patient Expanded Access Applications: Form FDA 3926; Draft Guidance for Industry

Dear Associate Commissioner Kux:

LLS appreciates this opportunity to submit comments on, and offer our support of, the Food & Drug Administration (FDA) Draft document entitled, "Individual Patient Expanded Access Applications: Form FDA 3926," which is intended to help streamline expanded access to investigational drugs.

As the world's largest voluntary organization dedicated to the needs of blood cancer patients, the Leukemia & Lymphoma Society is a strong supporter of initiatives that will help facilitate the discovery, development and delivery of innovative therapies and diagnostics for blood cancer patients. LLS also supports the use of expanded access by patients with serious diseases for which no effective therapies are available, and who cannot obtain the investigational drug under another open IND or clinical protocol. Expanded access programs provide these patients with the opportunity to access potentially life-saving therapies.

LLS is committed to supporting patients along the continuum of their disease journey with the multiple challenges they encounter because of their diagnosis. This support includes a variety of different programs and services not least of which is providing patients with the most up-to-date information on available treatment options for their specific diagnosis. For many of these patients the best available treatment option is enrolling in an active clinical trial. However, for those patients who do not qualify for enrollment in a clinical trial, and who may be unresponsive to current treatment options, or for whom an approved treatment is not available, LLS provides the support necessary to help patients understand and navigate the expanded access program and process.

LLS is very pleased that the FDA has simplified the individual patient expanded access application form (Form FDA 3926). This form is an important first step in simplifying the process for helping patients gain access to potentially lifesaving therapies. While an important first step, LLS believes that providers and patients would benefit from additional clarity around certain issues in the guidance.

LLS believes that the requirements to use Form FDA 1571 for subsequent submissions are potentially confusing. We request that the final guidance clarify when Form FDA 1571 should be used for subsequent submissions to an individual expanded access request, and that the FDA provide examples of situations that might require the use of Form FDA 1571. For example, the guidance might describe the type of treatment modifications or events that require additional submissions. This additional detail will help treating physicians comply with all of the regulatory requirements and avoid unnecessary delays.

LLS's mission is to cure leukemia, lymphoma, Hodgkin's disease and myeloma and improve the quality of life of patients and their families. We advocate on behalf of all blood cancer patients to ensure they have sustainable access to quality, effective, affordable, coordinated healthcare. LLS has provided more than $1 billion for research aimed at discovering, developing and delivering blood cancer cures since its founding. LLS-funded research has been part of nearly all of the FDA-approved therapies for blood cancer treatment.

LLS would like to thank the FDA's for their continued work and outreach efforts regarding this issue, and other issues of importance to patients. We appreciate this opportunity to represent the voice blood cancer patients to the FDA and look forward to working with you on this, and other issues of importance to LLS and the patients whom we serve. If you have any questions please do not hesitate to contact me directly at 202-969-1810 or bernadette.odonoghue@lls.org.

Sincerely,

Bernadette O'Donoghue

Bernadette O'Donoghue
Executive Director, Policy Development & Regulatory Affairs
The Leukemia & Lymphoma Society