LEUKEMIA &
LYMPHOMA
SOCIETY®
fighting blood cancers

www.lls.org

August 5, 2013

Leslie Kux
Assistant Commissioner for Policy
Food and Drug Administration
10903 New Hampshire Avenue
Silver Spring, Maryland 20993

Re: Availability of Anonymized Non-Summary Safety and Efficacy Data; Request for Comments, FDA-2013-N-0271

Filed Electronically at http://www.regulations.gov

Dear Ms. Kux:

The Leukemia & Lymphoma Society (LLS) is pleased to provide comments in response to the Availability of Anonymized Non-Summary Safety and Efficacy Data; Request for Comments.

LLS is the world's largest voluntary health agency dedicated to the needs of blood cancer patients. 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, Hodgkin's disease and myeloma and to ensure that blood cancer patients have sustainable access to quality, affordable, coordinated healthcare. LLS funds lifesaving blood cancer research around the world, provides free information and support services, and advocates for public policies that address the needs of patients with blood cancer. Since our founding 65 years ago, LLS has invested nearly $1 billon into research for cures.

LLS supports the FDA's goal of improving the efficiency and effectiveness of medical product development. LLS agrees that the availability of de-identified and masked clinical and preclinical data derived from marketing applications will make an important contribution toward this goal. This scientific data will assist in the generation of new knowledge and will facilitate innovation in the development of critically needed medical products.

LLS agrees that the contribution of patients who participate in clinical trials should be maximized for the benefit of society. However, this contribution should not come at the sacrifice of the patients' privacy, and therefore we believe that any effort to provide masked and de-identified data should make patients' privacy paramount.

LLS also believes that any effort by the FDA to make such masked and de-identified data available must not come at the expense of the FDA's core regulatory mission. Resources needed for the evaluation of the new therapies should not be diverted in order to process requests for masked and de-identified data.

Office of Public Policy
10 G Street NE, Suite 501, Washington, DC 20002 | *tel.* 202.408.7631 | *fax.* 202.408.7638

FDA requested responses to several questions regarding making anonymized non-summary data available. LLS addresses several of those questions below.

(1) What factors should be considered in masking study data (e.g., data fields from regulatory submissions to remove or modify, number of different products to pool within a product class)?

The Agency should consider efforts to make aggregating or pooling data as efficient as possible. This may require new considerations in how data is submitted to the FDA, including greater standardization of how data is submitted, as increased standardization may simplify the masking of data. As stated earlier, LLS is concerned that efforts to provide masked and de-indentified data has the potential to distract from the Agency's core mission. All efforts to prevent the diversion of resources must be considered.

(2) What limitations, if any, should there be on the Agency's ability to make available the masked data as described previously?

In considering limitations on masked and de-indentified data, the Agency should focus on issues related to protecting patient privacy and supporting innovation.

The Agency should manage to whom it makes masked data available. The Agency should only provide masked data to those persons performing responsible research – including academics, non-profits and industry – that agree to use the data in the advancement of drug safety or efficacy research. Requesting persons should be required to take all steps necessary to protect patient privacy.

The Agency should develop explicit regulation or guidance describing how and to whom masked data will be provided. Such regulation or guidance should require the Agency be aware of how the party intends to use the data, have in place restrictions regarding with whom the party requesting the data can share the data, and have in place requirements that parties do not try to re-identify the data. Penalties for violating these requirements should be explicitly stated.

Furthermore, given the limited resources at the FDA, the Agency should consider limiting access to masked data to specific requests to advance regulatory science. For example, valid requests could include requests for data to validate biomarkers, to examine specific primary or secondary endpoints, or to otherwise model disease progression in control arms.

(4) Would regulatory changes facilitate implementation of such a proposal, and if so, what changes would be most useful?

If the FDA chooses to permit masked and de-identified data to be made available, the Agency should require that notification and informed consent documentation be provided to patients enrolled in clinical trials. While de-identified data is unlikely to expose patient personal information to outside parties, the risk that patient data may be "re-identified" exists, and therefore patients should be made aware of this potential. LLS recognizes that an "opt-out" form may not be feasible (i.e., since one opt-out might eliminate information from an entire study), but patients choosing to participate in a clinical trial should be made aware that their de-identified information may become part of a larger data set.

The FDA might also consider requiring that "pre-masked" data sets be included alongside regulatory submissions. In an effort to make the data submitted to the FDA as easy to pool as possible, FDA should consider the creation of a pilot program to determine how sponsors can assist the Agency in preparing data for pooling.

Finally, the Agency should consider giving sponsors the option to opt-out of masking data. While de-identification of patient data must occur, permitting companies to "opt-out" of masking data could eliminate some of the cost and time constraints on the Agency. Furthermore, allowing companies to opt-out of masking data may further encourage independent efforts to pool data.

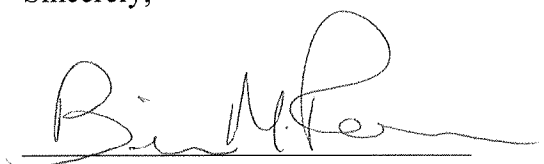
(5) Which situations do you believe disclosing masked data would be most useful to advance public health?

LLS believes that disclosing masked data will be extremely useful in the development of the novel precision medicines being developed to treat hematological malignancies. Given the small subpopulations of patients in each disease state, it can be difficult for researchers to accrue large randomized trials. Providing researchers with another resource to explore and validate biomarkers and other data sets that are otherwise difficult to build without large public data sets will have significant utility.

Providing masked data may also be useful in the creation of virtual control groups in situations where there are ethical considerations in providing only placebo treatment. While such data will not take the place of proper clinical trial procedures, it may assist researchers by giving them additional comparators and tools in the development of clinical trials.

Once again, LLS appreciates this opportunity to comment and looks forward to working with the FDA in our mutual effort to ensure that patients have access to the safest, most effective, and most innovative treatments available.

Sincerely,



Brian Rosen
Senior Vice President, Public Policy
The Leukemia & Lymphoma Society