



August 20, 2021

Janet Woodcock, MD  
Acting Commissioner  
U.S. Food and Drug Administration  
10903 New Hampshire Avenue  
Silver Spring, MD 20993

Re: Draft Guidance for Industry – *Cancer Clinical Trial Eligibility Criteria: Available Therapy in Non-Curative Settings*

Dear Dr. Woodcock:

The American Society of Hematology (ASH) appreciates the opportunity to provide comments to the U.S. Food and Drug Administration (FDA) on the Agency's *Cancer Clinical Trial Eligibility Criteria: Available Therapy in Non-Curative Settings*.

ASH represents more than 18,000 clinicians and scientists worldwide, who are committed to the study and treatment of blood and blood-related diseases. These disorders encompass malignant hematologic disorders such as leukemia, lymphoma, and multiple myeloma, as well as non-malignant conditions such as sickle cell disease, thalassemia, bone marrow failure, venous thromboembolism, and hemophilia. In addition, hematologists are pioneers in demonstrating the potential of treating various hematologic diseases and continue to be innovators in the field of stem cell biology, regenerative medicine, transfusion medicine, and gene therapy. ASH membership is comprised of basic, translational, and clinical scientists, as well as physicians providing care to patients.

In 2018, ASH founded the ASH Research Collaborative (ASH RC) to foster collaborative partnerships that accelerate progress in hematology, with the goal of improving the lives of people affected by blood diseases. The foundation of the ASH RC is its Data Hub and Sickle Cell Disease Clinical Trials Network. The Data Hub is a technology platform that facilitates the exchange of information by aggregating research-grade data on hematologic diseases. The core of the ASH RC programs is focused on patient centeredness and inclusion.

The Society is pleased to provide comments on the Draft Guidance. Overall, the Society is supportive of this document and the Agency's goal of increasing the inclusion of cancer patients (especially those with incurable cancers or individuals with hematologic malignancies with unfavorable long-term overall survival) in clinical trials. ASH agrees that cancer clinical trials should expand eligibility criteria to include these patients as long as the patients:

- have been provided appropriate informed consent that clearly states other treatment options might be clinically beneficial to them; and
- have discussed and understand the possible benefits, risks, and uncertainties associated with the drug being studied.

ASH further agrees with the Agency's recommendation that if and when such patients are enrolled into a study, they could be evaluated as a separate cohort to effectively interpret the efficacy of the drug being studied.

**2021**

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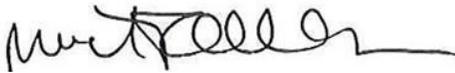
Martha Liggett, Esq.

ASH is supportive of these proposed recommendations because they will allow hematologists to recommend the best possible treatment path for their patients, which could be participating in a clinical trial rather than being required to first use existing treatments that might be suboptimal for them. This is important because in some hematologic malignancy trials exposing patients to available, but not curable or marginally effective treatments may result in patients being ineligible to participate in clinical trials later. This is due to poor performance status or residual toxicities, particularly cytopenias. Overall, this guidance ensures all cancer patients are involved in the decision-making process of their care, and understand the potential risks, benefits, and uncertainties of choosing a product in clinical development over a standard therapy, which may in general offer benefits to overall survival.

Additionally, we hope that the FDA will consider developing a similar guidance for non-malignant diseases as our constituency would benefit from a similar resource for other hematologic diseases.

Again, ASH appreciates the opportunity to provide these comments. Please consider ASH as a resource; we would be pleased to provide additional information or support. If you have any questions, please use ASH Deputy Director of Government Relations and Public Health, Stephanie Kaplan ([skaplan@hematology.org](mailto:skaplan@hematology.org) or 202-776-0544) as your point of contact.

Sincerely,

A handwritten signature in black ink, appearing to read "Martin S. Tallman", with a long horizontal flourish extending to the right.

Martin S. Tallman, MD  
President