



December 7, 2018

Dockets Management Staff (HFA-305)
Food and Drug Administration
5630 Fishers Lane, Rm. 1061
Rockville, MD 20852

Submitted via: <http://www.regulations.gov>

Re: Docket No. FDA-2018-D-2173, “Long Term Follow-Up After Administration of Human Gene Therapy Products; Draft Guidance for Industry”

Dear Dockets Manager:

AABB appreciates the opportunity to submit comments in response to the notice of availability for the [“Long Term Follow-Up After Administration of Human Gene Therapy Products; Draft Guidance for Industry”](#) guidance document, which was published in the Federal Register on July 12, 2018.

AABB is an international, not-for-profit association representing institutions and individuals involved in transfusion medicine, cellular therapies and patient blood management. The association is committed to “making transfusion medicine and cellular therapies safe, available and effective worldwide.” AABB works toward this vision by developing and delivering standards, accreditation, and educational programs for professionals that focus on optimizing patient and donor care and safety. AABB membership includes physicians, nurses, scientists, researchers, administrators, medical technologists, and other health care providers.

The information provided in the draft guidance document is helpful in guiding sponsors and investigators in assessing the need for long term follow-up (LTFU) based on risk for patients who have received gene therapy products. Not all gene therapy trial products will move on to licensure. We are requesting clarification or expansion of the following items within the draft guidance.

1. As noted in Section VII, the study sponsor, either corporate or academic principal investigator, may cease business operations or leave the organization as the case may be. In these cases, the sponsor is directed to consult with OTAT. We ask the agency to be mindful that requiring the institution that administered the gene therapy product to be responsible for tracking and submitting long term follow up data could be overly burdensome and at times, not possible.
2. As noted in lines 1075-1083 (Section VI), “Additional pharmacovigilance elements may be needed... For instance, we may recommend that you establish a registry to systematically capture and track data from treated patients with solicited sample collection, and follow-up of adverse events to resolution or stabilization to collect additional pertinent data. It may be necessary to establish a registry system to specifically capture adverse event data from treated patients who receive a GT product. This registry system can be a part of the PVP plan and

4550 Montgomery Avenue
Suite 700, North Tower
Bethesda, MD 20814-3304
+ 1 301.907.6977 MAIN
+ 1 301.907.6895 FAX
www.aabbccct.org



Advancing Transfusion and
Cellular Therapies Worldwide



reviewed at the time of licensure.” We respectfully request further clarification about the specific role a registry could play in providing LTFU data to the FDA, not only post-marketing but also during the pre-market phase of the clinical trials.

3. Regarding the requirement for investigators to maintain a record of exposures to mutagenic agents [Section V(D.)], many of these patients have malignancies or other diseases which warrant treatment with agents that are considered mutagenic. Distinguishing which agent caused which “new condition” may be quite challenging and we appreciate that the agency understands this and is willing to work with sponsors to determine the source in these complex clinical settings.

This is a very thoughtful, well laid out guidance document that provides important information for responsible parties to develop LTFU procedures for their patients. We commend the agency on the document and its format, including the use of flow charts.

Thank you for the opportunity to submit comments. Should you have any questions regarding these comments or would like additional information, please contact Kathy Loper, Senior Director, Center for Cellular Therapies at kloper@aabb.org.

Sincerely,

Kathy Loper, MHS, MT(ASCP)
Senior Director, Cellular Therapies
AABB Center for Cellular Therapies
www.aabcct.org
301.215.6558

4550 Montgomery Avenue
Suite 700, North Tower
Bethesda, MD 20814-3304
+ 1 301.907.6977 MAIN
+ 1 301.907.6895 FAX
www.aabcct.org