4164-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2013-D-0576]

Considerations for the Design of Early-Phase Clinical Trials of Cellular and Gene Therapy Products; Guidance for Industry; Availability

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice.

SUMMARY: The Food and Drug Administration (FDA or Agency) is announcing the availability of a document entitled "Considerations for the Design of Early-Phase Clinical Trials of Cellular and Gene Therapy Products; Guidance for Industry." The guidance document is to assist sponsors and investigators in designing early-phase clinical trials for cellular therapy (CT) and gene therapy (GT) products (referred to collectively as CGT products). The guidance document provides recommendations regarding clinical trials in which the primary objectives are the initial assessments of safety, tolerability, or feasibility of administration of investigational products. The guidance announced in this notice finalizes the draft guidance of the same title dated July 2013.

DATES: Submit either electronic or written comments on Agency guidances at any time.

ADDRESSES: Submit written requests for single copies of the guidance to the Office of Communication, Outreach, and Development, Center for Biologics Evaluation and Research (CBER), Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 71, rm. 3128,

Silver Spring, MD 20993-0002. Send one self-addressed adhesive label to assist the office in processing your requests. The guidance may also be obtained by mail by calling CBER at 1-800-835-4709 or 240-402-7800. See the SUPPLEMENTARY INFORMATION section for electronic access to the guidance document.

Submit electronic comments on the guidance to http://www.regulations.gov. Submit written comments to the Division of Dockets Management (HFA-305), Food and Drug Administration, 5630 Fishers Lane, rm. 1061, Rockville, MD 20852.

FOR FURTHER INFORMATION CONTACT: Valerie Butler, Center for Biologics Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 71, rm. 7301, Silver Spring, MD 20993-0002, 240-402-7911.

SUPPLEMENTARY INFORMATION:

I. Background

FDA is announcing the availability of a document entitled "Considerations for the Design of Early-Phase Clinical Trials of Cellular and Gene Therapy Products; Guidance for Industry." The guidance document is to assist sponsors and investigators in designing early-phase clinical trials for CGT products. The document provides recommendations regarding clinical trials in which the primary objectives are the initial assessments of safety, tolerability, or feasibility of administration of investigational products. The scope of the guidance is limited to products for which the Office of Cellular, Tissue, and Gene Therapies/Center for Biologics Evaluation and Research/FDA has regulatory authority. CGT products within the scope of the guidance meet the definition of ''biological product'' in section 351(i) of the Public Health Service (PHS) Act (42 U.S.C. 262(i)) and include CT and GT products that are used as therapeutic vaccines. The guidance does not apply to those human cells, tissues, and cellular- and tissue-based products

(HCT/Ps) regulated solely under section 361 of the PHS Act (42 U.S.C. 264), or to products regulated as medical devices under the Federal Food, Drug, and Cosmetic Act, or to the therapeutic biological products for which the Center for Drug Evaluation and Research has regulatory responsibility.

The design of early-phase clinical trials of CGT products often differs from the design of clinical trials for other types of pharmaceutical products. Differences in trial design are necessitated by the distinctive features of these products, and also may reflect previous clinical experience. The guidance document describes features of CGT products that influence clinical trial design, including product characteristics, manufacturing considerations, and preclinical considerations, and suggests other documents for additional information. Consequently, the guidance document provides recommendations with respect to these products as to clinical trial design, including early phase trial objectives, choosing a study population, using a control group and blinding, dose selection, treatment plans, monitoring, and follow-up. Finally, the guidance encourages prospective sponsors to meet with FDA review staff regarding their investigational new drug application (IND) submission and offers references for additional guidance on submitting an IND.

In the Federal Register of July 2, 2013 (78 FR 39736), FDA announced the availability of the draft guidance of the same title dated July 2013. FDA requested that comments on the guidance be submitted by November 22, 2013. In the Federal Register of November 20, 2013 (78 FR 69690), FDA extended the comment period for the draft guidance to May 9, 2014, to provide interested persons additional time to submit comments and to allow for public discussion of the draft guidance document at the Cellular, Tissue, and Gene Therapies Advisory Committee

meeting, which was ultimately held on February 25-26, 2014 (78 FR 79699, December 31, 2013).

FDA received a number of comments on the draft guidance and these comments were considered as the guidance was finalized. In addition, editorial changes were made to improve clarity. The guidance announced in this notice finalizes the draft guidance of the same title dated July 2013.

The guidance is being issued consistent with FDA's good guidance practices regulation (21 CFR 10.115). The guidance represents FDA's current thinking on considerations for the design of early-phase clinical trials of cellular and gene therapy products. It does not create or confer any rights for or on any person and does not operate to bind FDA or the public. An alternative approach may be used if such approach satisfies the requirements of the applicable statutes and regulations.

II. Paperwork Reduction Act of 1995

This guidance refers to previously approved collections of information found in FDA regulations. These collections of information are subject to review by the Office of Management and Budget (OMB) under the Paperwork Reduction Act of 1995 (44 U.S.C. 3501-3520). The collections of information in 21 CFR part 312 have been approved under OMB control number 0910-0014.

III. Comments

Interested persons may submit either electronic comments regarding this document to http://www.regulations.gov or written comments to the Division of Dockets Management (see ADDRESSES). It is only necessary to send one set of comments. Identify comments with the docket number found in brackets in the heading of this document. Received comments may be

seen in the Division of Dockets Management between 9 a.m. and 4 p.m., Monday through Friday, and will be posted to the docket at http://www.regulations.gov.

IV. Electronic Access

Persons with access to the Internet may obtain the guidance at either http://www.fda.gov/BiologicsBloodVaccines/GuidanceComplianceRegulatoryInformation/Guidances/default.htm or http://www.regulations.gov.

Dated: June 5, 2015.

Leslie Kux,

Associate Commissioner for Policy.

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