



DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2009-D-0490]

Draft Guidance for Industry and FDA Staff: Investigational New Drug Applications for Minimally Manipulated, Unrelated Allogeneic Placental/Umbilical Cord Blood Intended for Hematopoietic and Immunologic Reconstitution in Patients with Disorders Affecting the Hematopoietic System; Availability

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice.

SUMMARY: The Food and Drug Administration (FDA) is announcing the availability of a draft document entitled "Guidance for Industry and FDA Staff: Investigational New Drug Applications for Minimally Manipulated, Unrelated Allogeneic Placental/Umbilical Cord Blood Intended for Hematopoietic and Immunologic Reconstitution in Patients with Disorders Affecting the Hematopoietic System" dated June 2013. The draft guidance document provides advice to potential sponsors, such as cord blood banks, registries, transplant centers, or individual physicians serving as sponsor-investigators, to assist in the submission of an Investigational New Drug Application (IND) for certain hematopoietic progenitor cells from placental/umbilical cord blood (HPC, Cord Blood), when such HPC, Cord Blood units are not licensed, and when a suitable human leukocyte antigen matched cord blood transplant is needed for hematopoietic and immunologic reconstitution in patients with disorders affecting the hematopoietic system that are inherited, acquired, or result from myeloablative treatment and there is no satisfactory alternative treatment available. If unlicensed HPC, Cord Blood units are

made available for clinical use, they must be distributed under an IND. The draft guidance, when finalized, is intended to supersede the document entitled "Guidance for Industry and FDA Staff: Investigational New Drug Applications (INDs) for Minimally Manipulated, Unrelated Allogeneic Placental/Umbilical Cord Blood Intended for Hematopoietic Reconstitution for Specified Indications" dated June 2011.

DATES: Although you can comment on any guidance at any time (see 21 CFR 10.115(g)(5)), to ensure that the Agency considers your comment on this draft guidance before it begins work on the final version of the guidance, submit either electronic or written comments on the draft guidance by [INSERT DATE 90 DAYS AFTER DATE OF PUBLICATION IN THE FEDERAL REGISTER].

ADDRESSES: Submit written requests for single copies of the draft guidance to the Office of Communication, Outreach and Development (HFM-40), Center for Biologics Evaluation and Research (CBER), Food and Drug Administration, 1401 Rockville Pike, suite 200N, Rockville, MD 20852-1448. Send one self-addressed adhesive label to assist the office in processing your requests. The draft guidance may also be obtained by mail by calling CBER at 1-800-835-4709 or 301-827-1800. See the SUPPLEMENTARY INFORMATION section for electronic access to the draft guidance document.

Submit electronic comments on the draft 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 A. Butler, Center for Biologics Evaluation and Research (HFM-17), Food and Drug Administration, 1401 Rockville Pike, suite 200N, Rockville, MD 20852-1448, 301-827-6210.

## SUPPLEMENTARY INFORMATION:

## I. Background

FDA is announcing the availability of a draft document entitled "Guidance for Industry and FDA Staff: Investigational New Drug Applications for Minimally Manipulated, Unrelated Allogeneic Placental/Umbilical Cord Blood Intended for Hematopoietic and Immunologic Reconstitution in Patients with Disorders Affecting the Hematopoietic System" dated June 2013. The draft guidance, when finalized, will provide advice to potential sponsors to assist in the submission of an IND for certain HPC, Cord Blood, when such HPC, Cord Blood units are not licensed in accordance with Title 21 Code of Federal Regulations Part 601 (21 CFR part 601), and when a suitable human leukocyte antigen matched cord blood transplant is needed for hematopoietic and immunologic reconstitution in patients with disorders affecting the hematopoietic system that are inherited, acquired, or result from myeloablative treatment and there is no satisfactory alternative treatment available. If unlicensed HPC, Cord Blood units are made available for clinical use, they must be distributed under an IND meeting the applicable requirements in 21 CFR part 312. The draft guidance, when finalized, is intended to supersede the document entitled "Guidance for Industry and FDA Staff: Investigational New Drug Applications (INDs) for Minimally Manipulated, Unrelated Allogeneic Placental/Umbilical Cord Blood Intended for Hematopoietic Reconstitution for Specified Indications" dated June 2011.

The draft guidance is being issued consistent with FDA's good guidance practices regulation (21 CFR 10.115). The draft guidance, when finalized, will represent FDA's current thinking on this topic. 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 requirement of the applicable statutes and regulations.

Elsewhere in this issue of the Federal Register, we also are announcing the availability of another, related draft guidance entitled "Guidance for Industry: Biologics License Applications for Minimally Manipulated, Unrelated Allogeneic Placental/Umbilical Cord Blood Intended for Hematopoietic and Immunologic Reconstitution in Patients with Disorders Affecting the Hematopoietic System." That draft guidance, when finalized, is intended to supersede the document entitled "Guidance for Industry: Minimally Manipulated, Unrelated Allogeneic Placental/Umbilical Cord Blood Intended for Hematopoietic Reconstitution for Specified Indications" dated October 2009.

## II. Paperwork Reduction Act of 1995

This draft 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; 21 CFR part 56 have been approved under OMB control number 0910-0130; 21 CFR part 1271 have been approved under OMB control number 0910-0543; and Form FDA 1571 has been approved under OMB control number 0910-0014.

## III. Comments

The draft guidance is being distributed for comment purposes only and is not intended for implementation at this time. 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 draft guidance at either <http://www.fda.gov/BiologicsBloodVaccines/GuidanceComplianceRegulatoryInformation/Guidances/default.htm> or <http://www.regulations.gov>.

Dated: June 10, 2013.

Leslie Kux,

Assistant Commissioner for Policy.