4160-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2012-D-1145]

Draft Guidance for Industry on Enrichment Strategies for Clinical Trials to Support Approval of Human Drugs and Biological Products; Availability

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice.

SUMMARY: The Food and Drug Administration (FDA) is announcing the availability of a draft guidance for industry entitled "Enrichment Strategies for Clinical Trials to Support Approval of Human Drugs and Biological Products." The purpose of this document is to provide guidance to industry on enrichment strategies that can be used in clinical trials intended to support effectiveness and safety claims in new drug applications (NDAs) and biologics license applications (BLAs). This document defines several types of enrichment strategies, provides examples of various potential clinical trial designs, and discusses potential regulatory considerations when using enrichment strategies in clinical trials.

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comment on any guidance at any time (see 21 CFR 10.115(g)(5)), to ensure that the Agency considers your comment on this draft guidance before it begins work on the final version of the guidance, submit either electronic or written comments on the draft guidance by [INSERT DATE 60 DAYS AFTER DATE OF PUBLICATION IN THE FEDERAL REGISTER].

ADDRESSES: Submit written requests for single copies of the draft guidance to the Division of Drug Information, Center for Drug Evaluation and Research, Food and Drug Administration,

10903 New Hampshire Ave., Bldg. 51, rm. 2201, Silver Spring, MD 20993-0002; or the Office of Communication, Outreach and Development (HFM-40), Center for Biologics Evaluation and Research, Food and Drug Administration, 1401 Rockville Pike, suite 200N, Rockville, MD 20852-1448 (1-800-835-4709 or 301-827-1800), or the Division of Small Manufacturers, International and Consumer Assistance, Center for Devices and Radiological Health, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 66, rm. 4613, Silver Spring, MD 20993-0002, or fax your request to 301-847-8149. Send one self-addressed adhesive label to assist that office in processing your requests. See the SUPPLEMENTARY INFORMATION section for electronic access to the draft guidance document.

Submit electronic comments on the draft guidance to http://www.regulations.gov.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:

Robert Temple,

Center for Drug Evaluation and Research,

Food and Drug Administration,

10903 New Hampshire Ave.,

Bldg. 22, rm. 4212,

Silver Spring, MD 20993-0003,

301-796-2270; or

Stephen Ripley,

Center for Biologics Evaluation and Research,

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1401 Rockville Pike,

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Rockville, MD 20852-1448,

301-827-6210; or

Robert L. Becker,

Center for Device and Radiological Health,

Food and Drug Administration,

10903 New Hampshire Ave.,

Bldg. 66, rm. 5674,

Silver Spring, MD 20993-0003,

301-796-5450.

SUPPLEMENTARY INFORMATION:

I. Background

FDA is announcing the availability of a draft guidance for industry entitled "Enrichment Strategies for Clinical Trials to Support Approval of Human Drugs and Biological Products." This document provides guidance to industry on enrichment strategies that can be used in clinical trials intended to support effectiveness and safety claims in new drug applications (NDAs) and biologics license applications (BLAs). Similar approaches could be used in clinical trials in earlier phases of drug development. As part of the reauthorization of the Prescription Drug User Fee Act (PDUFA IV), FDA committed to certain performance goals (see letters from the Secretary of Health and Human Services to the Chairman of the Committee on Health, Education, Labor, and Pensions of the Senate and the Chairman of the Committee on Energy and

Commerce of the House of Representatives, as set forth in the Congressional Record).[1] This draft guidance addresses one of these goals with the creation of a guidance document that addresses enriched trial designs. The guidance defines and discusses three enrichment strategies: Decreasing heterogeneity, predictive enrichment, and prognostic enrichment. The guidance also discusses general clinical trial design considerations, provides examples of potential clinical trial designs, and discusses regulatory considerations when using enrichment strategies.

This draft guidance is being issued consistent with FDA's good guidance practices regulation (21 CFR 10.115). The draft guidance, when finalized, will represent the Agency's current thinking on clinical trial designs employing enrichment strategies to support approval of human drugs and biological 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. Comments

Interested persons may submit to the Division of Dockets Management (see ADDRESSES) either electronic or written comments regarding this document. 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.

[1] See "Section A: PDUFA Reauthorization Performance Goals and Procedures Fiscal Years 2008 Through 2012" (http://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm119243.htm).

III. Electronic Access

Persons with access to the Internet may obtain the document at either http://www.fda.gov/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/default.htm, http://www.fda.gov/BiologicsBloodVaccines/GuidanceComplianceRegulatoryInformation/default.htm, http://www.fda.gov/MedicalDevices/DeviceRegulationandGuidance/GuidanceDocuments/default.htm, or http://www.regulations.gov.

Dated: November 20, 2012.

Leslie Kux,

Assistant Commissioner for Policy.

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