4164-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2015-D-1246]

Investigational Enzyme Replacement Therapy Products: Nonclinical Assessment; Draft Guidance for Industry; Availability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SUMMARY: The Food and Drug Administration (FDA or Agency) is announcing the availability of a draft guidance for industry entitled "Investigational Enzyme Replacement Therapy Products: Nonclinical Assessment." This draft guidance is intended to advise the sponsors and individuals involved in the design and implementation of nonclinical studies on the substance and scope of nonclinical information needed to support first-in-human clinical trials, ongoing clinical development, and eventual approval of enzyme replacement therapy (ERT) products for the treatment of rare, life-threatening conditions.

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comment on any guidance at any time (see 21 CFR 10.115(g)(5)), to ensure that the Agency considers your comment on this draft guidance before it begins work on the final version of the guidance, submit either electronic or written comments on the draft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ADDRESSES: Submit written requests for single copies of the draft guidance to the Division of Drug Information, Center for Drug Evaluation and Research, Food and Drug Administration, 10001 New Hampshire Ave., Hillandale Building, 4th Floor, Silver Spring, MD 20993-0002. 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 See the SUPPLEMENTARY INFORMATION section for electronic access to the draft guidance document.

Submit electronic comments on the draft guidance to http://www.regulations.gov.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: Sushanta Chakder, Center for Drug Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 22, rm. 5108, Silver Spring, MD 20993-0002, 301-796-0861.

SUPPLEMENTARY INFORMATION:

I. Background

FDA is announcing the availability of a draft guidance for industry entitled "Investigational Enzyme Replacement Therapy Products: Nonclinical Assessment."

This draft guidance provides sponsors and individuals involved in the design and implementation of nonclinical studies with recommendations on the nonclinical information needed to support initiation of clinical trials, ongoing clinical development, and eventual

licensure or approval for investigational ERT products. The recommendations in this guidance are applicable to ERT products indicated for lysosomal storage diseases or other diseases related to inborn errors of metabolism.

Because of the wide array of clinical indications, natural history of disease, and product types, no single nonclinical program can be designed to address all ERT products, and a case-by-case approach to both toxicological evaluation and clinical development is warranted to optimize and expedite drug development. Common nonclinical issues, such as the number of animal species needed for safety assessment, selection of animal models and duration of the toxicology studies needed to support first-in-human trials, and nonclinical study requirements for ultimate licensure or market approval of the ERT product, are addressed in this guidance.

This guidance is intended as an adjunct to the ICH guidances for industry entitled "M3(R2) Nonclinical Safety Studies for the Conduct of Human Clinical Trials and Marketing Authorization for Pharmaceuticals," "M3(R2) Nonclinical Safety Studies for the Conduct of Human Clinical Trials and Marketing Authorization for Pharmaceuticals--Questions and Answers," and "S6 Preclinical Safety Evaluation of Biotechnology-Derived Pharmaceuticals."

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 current thinking of FDA on nonclinical assessment of investigational ERT products. It does not establish any rights for any person and is not binding on FDA or the public. You can use an alternative approach if it satisfies the requirements of the applicable statutes and regulations.

II. The Paperwork Reduction Act of 1995

This guidance refers to previously approved collections of information that 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, and the information collection in the regulations on good laboratory practice for nonclinical laboratory studies (21 CFR part 58) is approved under OMB control number 0910-0119.

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 document at either http://www.fda.gov/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/default.htm or http://www.regulations.gov.

Dated: May 7, 2015.

Leslie Kux,

Associate Commissioner for Policy.

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