4164-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2013-D-1275]

General Clinical Pharmacology Considerations for Pediatric Studies for Drugs and Biological Products; Draft Guidance for Industry; Availability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SUMMARY: The Food and Drug Administration (FDA) is announcing the availability of a draft guidance for industry entitled "General Clinical Pharmacology Considerations for Pediatric Studies for Drugs and Biological Products." The draft guidance is intended to assist those sponsors of new drug applications (NDAs), biologics license applications (BLAs) for therapeutic biologics, and supplements to such applications who are planning to conduct clinical studies in pediatric populations. Effectiveness, safety, or dose finding studies in pediatric patients involve gathering clinical pharmacology information, such as information regarding a product's pharmacokinetics and pharmacodynamics pertaining to dose selection and individualization. This draft guidance addresses general clinical pharmacology considerations for conducting studies so that the dosing and safety information for drugs and biologic products can be sufficiently characterized, leading to well-designed trials to evaluate effectiveness.

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on any guidance at any time (see 21 CFR 10.115(g)(5)), to ensure that the Agency considers your comment on this draft guidance before it begins work on the final version of the guidance, submit either electronic or written comments on the draft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ADDRESSES: Submit written requests for single copies of the draft guidance to the Division of Drug Information, Center for Drug Evaluation and Research, Food and Drug Administration, 10001 New Hampshire Ave., Hillandale Building, 4th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 See the SUPPLEMENTARY INFORMATION section for electronic access to the draft guidance document.

Submit electronic comments on the draft guidance to http://www.regulations.gov.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: Gilbert J. Burckart, Center for Drug Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 51, rm. 3184, Silver Spring, MD 20993-0002, 301-796-2065.

SUPPLEMENTARY INFORMATION:

I. Background

FDA is announcing the availability of a draft guidance for industry entitled "General Clinical Pharmacology Considerations for Pediatric Studies for Drugs and Biological Products." During the past two decades, FDA has worked to address the problem of inadequate pediatric testing and inadequate pediatric use information in drug and biological product labeling. The Food and Drug Administration Modernization Act of 1997 (Public Law 105-115) addressed the need for improved information about drug use in the pediatric population (codified at 21 U.S.C. 355a) by establishing incentives for conducting pediatric studies on drugs while exclusivity or

patent protection exists. Congress subsequently passed the Best Pharmaceuticals for Children Act (BPCA) in 2002 and the Pediatric Research Equity Act (PREA) in 2003. Both BPCA and PREA were reauthorized in 2007 and were made permanent under Title V of the Food and Drug Administration Safety and Innovation Act of 2012 (Public Law 112-144).

Under BPCA, sponsors of certain applications and supplements filed under section 505 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355) (the FD&C Act) can obtain an additional 6 months of exclusivity if, in accordance with the requirements of the statute, the sponsor submits information responding to a written request from the Secretary relating to the use of a drug in the pediatric population.

Under PREA, sponsors of certain applications and supplements filed under section 505 of the FD&C Act or section 351 of the Public Health Service Act are required to submit pediatric assessments, unless they receive an applicable waiver or deferral of this requirement. If applicable, sponsors must submit a request for a deferral or waiver as part of an initial pediatric study plan.

This draft guidance focuses on the clinical pharmacology information (e.g., exposure-response, pharmacokinetics, and pharmacodynamics) needed to support findings of effectiveness and safety and helps identify appropriate doses in pediatric populations. The draft guidance also describes the use of quantitative approaches (i.e., pharmacometrics) to employ disease and exposure-response knowledge from relevant prior clinical studies to design and evaluate future pediatric studies. The draft guidance does not describe: (1) Standards for approval of drugs and biological products in the pediatric population, (2) criteria to allow a determination that the course of a disease and the effects of a drug or a biologic are the same in adults and pediatric

populations, or (3) clinical pharmacology studies for vaccine therapy, blood products, or other products not regulated by the Center for Drug Evaluation and Research.

This draft guidance is being issued consistent with FDA's good guidance practices regulation (21 CFR 10.115). The draft guidance represents the Agency's current thinking on the general clinical pharmacology considerations for pediatric studies for 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 requirement of the applicable statutes and regulations.

II. Paperwork Reduction Act of 1995

This draft guidance includes information collection provisions that are subject to review by the Office of Management and Budget (OMB) under the Paperwork Reduction Act (PRA) of 1995 (44 U.S.C. 3501-3520) (PRA). The collections of information referenced in this draft guidance that are related to the burden for the submission of investigational new drug applications are covered under 21 CFR part 312 and have been approved under OMB control number 0910-0014. The collections of information referenced in this draft guidance that are related to the burden for the submission of new drug applications are covered under 21 CFR part 314 and have been approved under OMB control number 0910-0001. The submission of prescription drug product labeling under 21 CFR 201.56 and 201.57 is approved under OMB control number 0910-0572.

In accordance with the PRA, prior to publication of any final guidance document, FDA intends to solicit public comment and obtain OMB approval for any information collections recommended in this guidance that are new or that would represent material modifications to those previously approved collections of information found in FDA regulations or guidances.

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 http://www.fda.gov/Drugs/GuidanceComplianceRegulatoryInformation/Guidances or http://www.regulations.gov.

Dated: December 2, 2014.

Leslie Kux,

Associate Commissioner for Policy.

[FR Doc. 2014-28716 Filed 12/08/2014 at 8:45 am; Publication Date: 12/09/2014]