



DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2019-D-4751]

Food and Drug Administration Reauthorization Act Implementation Guidance for Pediatric Studies of Molecularly Targeted Oncology Drugs; Draft Guidance for Industry; Availability

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice of availability.

SUMMARY: The Food and Drug Administration (FDA or Agency) is announcing the availability of a draft guidance for industry entitled “FDARA Implementation Guidance for Pediatric Studies of Molecularly Targeted Oncology Drugs.” This draft guidance addresses early planning for pediatric evaluation of certain molecularly targeted oncology drugs, including biological products, for which original new drug applications (NDAs) and biologics license applications (BLAs) are expected to be submitted to FDA on or after August 18, 2020, in accordance with the provisions of the Federal Food, Drug, and Cosmetic Act (FD&C Act) as amended by the FDA Reauthorization Act of 2017 (FDARA). This guidance addresses the implementation of amendments made by FDARA to the FD&C Act regarding molecularly targeted oncology drugs.

DATES: Submit either electronic or written comments on the draft guidance by **[INSERT DATE 60 DAYS AFTER DATE OF PUBLICATION IN THE *FEDERAL REGISTER*]** to ensure that the Agency considers your comment on this draft guidance before it begins work on the final version of the guidance.

ADDRESSES: You may submit comments on any guidance at any time as follows:

Electronic Submissions

Submit electronic comments in the following way:

- Federal eRulemaking Portal: <https://www.regulations.gov>. Follow the instructions for submitting comments. Comments submitted electronically, including attachments, to <https://www.regulations.gov> will be posted to the docket unchanged. Because your comment will be made public, you are solely responsible for ensuring that your comment does not include any confidential information that you or a third party may not wish to be posted, such as medical information, your or anyone else's Social Security number, or confidential business information, such as a manufacturing process. Please note that if you include your name, contact information, or other information that identifies you in the body of your comments, that information will be posted on <https://www.regulations.gov>.
- If you want to submit a comment with confidential information that you do not wish to be made available to the public, submit the comment as a written/paper submission and in the manner detailed (see "Written/Paper Submissions" and "Instructions").

Written/Paper Submissions

Submit written/paper submissions as follows:

- Mail/Hand delivery/Courier (for written/paper submissions): Dockets Management Staff (HFA-305), Food and Drug Administration, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852.

- For written/paper comments submitted to the Dockets Management Staff, FDA will post your comment, as well as any attachments, except for information submitted, marked and identified, as confidential, if submitted as detailed in “Instructions.”

Instructions: All submissions received must include the Docket No. FDA-2019-D-4751 for “FDARA Implementation Guidance for Pediatric Studies of Molecularly Targeted Oncology Drugs.” Received comments will be placed in the docket and, except for those submitted as “Confidential Submissions,” publicly viewable at <https://www.regulations.gov> or at the Dockets Management Staff between 9 a.m. and 4 p.m., Monday through Friday.

- Confidential Submissions--To submit a comment with confidential information that you do not wish to be made publicly available, submit your comments only as a written/paper submission. You should submit two copies total. One copy will include the information you claim to be confidential with a heading or cover note that states “THIS DOCUMENT CONTAINS CONFIDENTIAL INFORMATION.” The Agency will review this copy, including the claimed confidential information, in its consideration of comments. The second copy, which will have the claimed confidential information redacted/blacked out, will be available for public viewing and posted on <https://www.regulations.gov>. Submit both copies to the Dockets Management Staff. If you do not wish your name and contact information to be made publicly available, you can provide this information on the cover sheet and not in the body of your comments and you must identify this information as “confidential.” Any information marked as “confidential” will not be disclosed except in accordance with 21 CFR 10.20 and other applicable disclosure law. For more information about FDA’s posting of comments to public dockets, see 80 FR 56469, September 18, 2015,

or access the information at: <https://www.gpo.gov/fdsys/pkg/FR-2015-09-18/pdf/2015-23389.pdf>.

Docket: For access to the docket to read background documents or the electronic and written/paper comments received, go to <https://www.regulations.gov> and insert the docket number, found in brackets in the heading of this document, into the “Search” box and follow the prompts and/or go to the Dockets Management Staff, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852.

You may submit comments on any guidance at any time (see 21 CFR 10.115(g)(5)). Submit written requests for single copies of the draft 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; Division of Drug Information, Center for Drug Evaluation and Research (CDER), 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. The draft guidance may also be obtained by mail by calling CBER at 1-800-835-4709 or 240-402-8010. See the SUPPLEMENTARY INFORMATION section for electronic access to the draft guidance document.

FOR FURTHER INFORMATION CONTACT: Gregory Reaman, Oncology Center of Excellence, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 22, Rm. 2202, Silver Spring, MD 20993-0002, 301-796-0785; or Stephen Ripley, 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 draft guidance for industry entitled “FDARA Implementation Guidance for Pediatric Studies of Molecularly Targeted Oncology Drugs: Amendments to Sec. 505B of the FD&C Act.” This draft guidance addresses early planning for pediatric evaluation of certain molecularly targeted oncology drugs (including biological products) for which original NDAs and BLAs are expected to be submitted to FDA on or after August 18, 2020, in accordance with the provisions of section 505B of the FD&C Act. Section 505B of the FD&C Act (21 U.S.C. 355c) (also referred to as the Pediatric Research Equity Act or PREA (Pub. L. 108-155)), was amended by FDARA.

The amendments provide a new mechanism to expedite the evaluation of certain novel drugs with the potential to address an unmet medical need of pediatric patients with cancer. Specifically, FDARA amended the requirement for pediatric investigations of certain new targeted cancer drugs to be based on molecular mechanism of action rather than clinical indication. For original NDAs and BLAs submitted on or after August 18, 2020, if the application is for a new active ingredient, and the drug or biological product that is the subject of the application is intended for treatment of an adult cancer and directed at a molecular target FDA determines to be substantially relevant to the growth or progression of a pediatric cancer, reports of molecularly targeted pediatric cancer investigations must be submitted with the marketing application, unless the required investigations are waived or deferred (section 505B(a)(1)(B) of the FD&C Act).

This draft guidance provides recommendations on regulatory considerations related to the amendments to section 505B of the FD&C Act, including information on molecular targets,

factors FDA intends to consider in the determination of whether a molecular target is substantially relevant to the growth or progression of a pediatric cancer, the molecular target lists, content of the initial pediatric study plan and description of recommended studies, additional considerations for rare cancers, and considerations for planned waivers and deferrals. In addition, the draft guidance includes information regarding global implications and international collaboration.

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 "FDARA Implementation Guidance for Pediatric Studies of Molecularly Targeted Oncology Drugs." 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. 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. 3501-3521). The collections of information in 21 CFR part 314 have been approved under OMB control number 0910-0001. The collections of information in 21 CFR part 312 have been approved under OMB control numbers 0910-0014. The collections of information in 21 CFR part 601 have been approved under OMB control number 0910-0338.

III. Electronic Access

Persons with access to the internet may obtain the draft guidance at either <https://www.fda.gov/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/default.htm>,

<https://www.fda.gov/BiologicsBloodVaccines/GuidanceComplianceRegulatoryInformation/Guidances/default.htm>, or <https://www.regulations.gov>.

Dated: December 9, 2019.

Lowell J. Schiller,

Principal Associate Commissioner for Policy.

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