4164-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2017-D-6380]

Clarification of Orphan Designation of Drugs and Biologics for Pediatric Subpopulations of Common Diseases; 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 final guidance for industry entitled "Clarification of Orphan Designation of Drugs and Biologics for Pediatric Subpopulations of Common Diseases." FDA does not expect to grant any additional orphan-drug designation to drugs for pediatric subpopulations of common diseases (i.e., diseases or conditions with an overall prevalence of 200,000 or greater). This will help resolve an unintended loophole in the Pediatric Research Equity Act (PREA) orphan exemption process where a sponsor holding a pediatric-subpopulation designation can submit a marketing application for use of its drug in the non-orphan adult population of that disease, get a pediatric-subpopulation designation for the pediatric subset of the disease, and, due to this designation, be exempt from conducting the pediatric studies normally required under PREA when seeking approval of the adult indication.

DATES: The announcement of the guidance is published in the *Federal Register* on [INSERT DATE OF PUBLICATION IN THE FEDERAL REGISTER].

ADDRESSES: You may submit either electronic or written comments on Agency guidances 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-2017-D-6380 for "Clarification of Orphan Designation of Drugs and Biologics for Pediatric Subpopulations of Common Diseases." 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 this guidance to the Office of Orphan Products Development, Office of the Commissioner, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 32, Rm. 5295, Silver Spring, MD 20993. Send one self-addressed adhesive label to assist that office in processing your requests. See the SUPPLEMENTARY INFORMATION section for electronic access to the guidance document.

FOR FURTHER INFORMATION CONTACT: Aaron Friedman, Office of Orphan Products Development, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 32, Rm. 5209, Silver Spring, MD 20993, 301-796-2989.

SUPPLEMENTARY INFORMATION:

I. Background

FDA is announcing the availability of a final guidance for industry entitled "Clarification of Orphan Designation of Drugs and Biologics for Pediatric Subpopulations of Common Diseases." In the *Federal Register* of December 20, 2017 (82 FR 60402), FDA published a notice of availability for the draft guidance entitled "Clarification of Orphan Designation of Drugs and Biologics for Pediatric Subpopulations of Common Diseases," announcing that FDA does not expect to grant any additional orphan drug designation to drugs for pediatric subpopulations of common diseases (i.e., diseases or conditions with an overall prevalence of

over 200,000 in the United States). In the *Federal Register* of January 12, 2018 (83 FR 1619), FDA announced that it was extending the comment period for this draft guidance for an additional 30 days. FDA received several comments on the draft guidance and those comments were considered as the guidance was finalized. The guidance announced in this notice finalizes the draft guidance dated December 2017. FDA does not expect to grant any additional orphan-drug designation to drugs for pediatric subpopulations of common diseases (i.e., diseases or conditions with an overall prevalence of 200,000 or greater). This will help resolve an unintended loophole in the Pediatric Research Equity Act (PREA) orphan exemption process where a sponsor holding a pediatric-subpopulation designation can submit a marketing application for use of its drug in the non-orphan adult population of that disease, get a pediatric-subpopulation designation for the pediatric subset of the disease, and, due to this designation, be exempt from conducting the pediatric studies normally required under PREA when seeking approval of the adult indication.

This guidance is being issued consistent with FDA's good guidance practices regulation (21 CFR 10.115). The guidance represents the current thinking of FDA on orphan designation of drugs and biologics for pediatric subpopulations of common diseases. 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. This guidance is not subject to Executive Order 12866.

II. Electronic Access

Persons with access to the internet may obtain the guidance at either https://www.fda.gov/Orphan or https://www.regulations.gov.

Dated: July 23, 2018.

Leslie Kux,

Associate Commissioner for Policy.

[FR Doc. 2018-16027 Filed: 7/26/2018 8:45 am; Publication Date: 7/27/2018]