4164-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2020-D-2199]

Investigational New Drug Submissions for Individualized Antisense Oligonucleotide Drug Products: Administrative and Procedural Recommendations; Draft Guidance for Sponsor-Investigators; Availability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SUMMARY: The Food and Drug Administration (FDA or Agency) is announcing the availability of a draft guidance for industry entitled "IND Submissions for Individualized Antisense Oligonucleotide Drug Products: Administrative and Procedural Recommendations." FDA is publishing this draft guidance to help sponsor-investigators (hereafter referred to as sponsors) developing individualized antisense oligonucleotide (ASO) drug products for a severely debilitating or life-threatening genetic disease. Most often, individuals with such diseases will not have adequate alternative therapy available for treating their disease. This draft guidance is intended to help sponsors of such development programs, who may be relatively unfamiliar with FDA regulations, processes, and practices, with the administrative and procedural aspects of interacting with FDA, including seeking feedback from FDA on their development programs and making regulatory submissions related to these development programs.

DATES: Submit either electronic or written comments on the draft guidance by [INSERT DATE 60 DAYS AFTER DATE OF PUBLICATION IN THE *FEDERAL REGISTER*] to ensure that the Agency considers your comment on this draft guidance before it begins work on the final version of the guidance.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-2020-D-2199 for "IND Submissions for Individualized Antisense Oligonucleotide Drug Products: Administrative and Procedural Recommendations." 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, 240-402-7500.

- 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.govinfo.gov/content/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, 240-402-7500.

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 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.

FOR FURTHER INFORMATION CONTACT: Colleen Locicero, Office of New Drugs, Center for Drug Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Avenue, Silver Spring, MD, 20903, 301-796-1114.

SUPPLEMENTARY INFORMATION:

I. Background

FDA is announcing the availability of a draft guidance for industry entitled "IND Submissions for Individualized Antisense Oligonucleotide Drug Products: Administrative and Procedural Recommendations." This draft guidance is intended to help sponsors developing individualized ASO drug products for a severely debilitating or life-threatening genetic disease.

The draft guidance addresses the approach for obtaining feedback from FDA, the expectations and process for making regulatory submissions to FDA, and high-level recommendations related to the requirement for institutional review board review of protocols for trials of individualized ASO drug products and the informed consent of participants. The draft guidance discusses the importance of early interaction with FDA, submission expectations for pre-investigational new drug (IND) meeting packages and IND applications, and ethical and human subject protection considerations.

The draft guidance is intended to help sponsors of such development programs, who may be relatively unfamiliar with FDA regulations, processes, and practices, seek feedback from FDA on their development programs and make regulatory submissions related to these development programs. The draft guidance is expected to facilitate the preparation of adequate

pre-IND and IND submissions for review by the Agency, which may help enable prompt initiation of the investigation.

This draft guidance represents the first of several guidances FDA intends to publish to advise and help sponsors developing individualized ASO drug products for patients who have severely debilitating or life-threatening diseases or conditions and no adequate alternative therapy available to them to treat their disease or condition.

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 "IND Submissions for Individualized Antisense Oligonucleotide Drug Products: Administrative and Procedural Recommendations." 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

While this guidance contains no collection of information, it does refer to previously approved FDA collections of information. Therefore, clearance by the Office of Management and Budget (OMB) under the Paperwork Reduction Act of 1995 (44 U.S.C. 3501-3521) is not required for this guidance. The previously approved collections of information are subject to review by OMB under the PRA. The collections of information in 21 CFR part 312 for the submission of IND applications, amendments, and safety reports; for investigator brochures; and for requesting a pre-IND meeting have been approved under OMB control number 0910-0014; the collections of information for paper submissions of Form FDA 3500A have been approved under OMB control number 0910-0291; the collections of information for electronic submissions of Form FDA 3500 have been approved under OMB control number 0910-0645; the collections of information in 21 CFR parts 50 and 56 for obtaining informed consent for prospective patients have been approved under OMB control number 0910-0755.

III. Electronic Access

Persons with access to the internet may obtain the draft guidance at either https://www.fda.gov/drugs/guidance-compliance-regulatory-information/guidances-drugs or https://www.regulations.gov.

Dated: December 23, 2020.

Lauren K. Roth,

Acting Principal Associate Commissioner for Policy.

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