4164-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2022-N-2480]

Rare Disease Endpoint Advancement Pilot Program Workshop: Novel Endpoints for Rare Disease Drug Development; Public Workshop; Request for Comments

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice of public workshop; request for comments.

SUMMARY: The Food and Drug Administration (FDA, the Agency, or we) is announcing a public workshop entitled "Rare Disease Endpoint Advancement Pilot Program Workshop: Novel Endpoints for Rare Disease Drug Development." Convened by the Duke-Robert J. Margolis, MD Center for Health Policy (Duke-Margolis) and supported by a cooperative agreement between FDA and Duke-Margolis, the workshop will include discussions of the Rare Disease Endpoint Advancement (RDEA) Pilot Program and novel endpoint development for rare disease drug development.

DATES: The public workshop will be held virtually on June 7, 2023, and June 8, 2023, from 1 p.m. to 5 p.m., Eastern Time. Either electronic or written comments on this public workshop must be submitted by July 23, 2023. See the **SUPPLEMENTARY INFORMATION** section for registration date and information.

ADDRESSES: The public workshop will be held virtually using the Zoom platform. The link for the public workshop will be sent to registrants upon registration.

You may submit comments as follows. Please note that late, untimely filed comments will not be considered. The https://www.regulations.gov electronic filing system will accept comments until 11:59 p.m. Eastern Time on July 23, 2023. Comments received by mail/hand delivery/courier (for written/paper submissions) will be considered timely if they are received on or before that date.

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-2022-N-2480 for "Rare Disease Endpoint Advancement Pilot Program Workshop: Novel Endpoints for Rare Disease Drug Development." Received comments, those filed in a timely manner (see

ADDRESSES), 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.

FOR FURTHER INFORMATION CONTACT: Mary Jo Salerno, Center for Drug Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Silver Spring, MD 20993, 240-402-0420, RDEA.Meetings@fda.hhs.gov.

SUPPLEMENTARY INFORMATION:

I. Background

This public workshop is intended to support the RDEA Pilot Program consistent with the requirements under section 3208 of the Food and Drug Omnibus Reform Act of 2022 (FDORA). Section 3208 of FDORA requires FDA to establish a pilot program to provide increased interaction with sponsors of rare disease drug development programs for purposes of advancing the development of efficacy endpoints, including surrogate and intermediate endpoints, for drugs intended to treat rare diseases. Section 3208 of FDORA also requires FDA to conduct up to three public workshops to discuss various topics relevant to the development of endpoints for rare diseases on or before September 30, 2026. This is the first of up to three public workshops to satisfy the FDORA requirement.

The public workshop is also intended to meet a performance goal under the FDA User Fee Reauthorization Act of 2022, in accordance with the PDUFA Reauthorization Performance Goals and Procedures Fiscal Years 2023 Through 2027 letter (PDUFA VII Commitment Letter), which is available at https://www.fda.gov/media/151712/download. Specifically, section I.K.4 of the PDUFA VII Commitment Letter, "Advancing Development of Drugs for Rare Diseases" (https://www.fda.gov/media/151712/download), outlines commitments, including up to three public workshops to discuss various topics relevant to endpoint development for rare diseases.

II. Topics for Discussion at the Public Workshop

The purpose of this public workshop is to: (1) provide an overview of the RDEA Pilot Program (a joint program of the Center for Drug Evaluation and Research and the Center for Biologics Evaluation and Research), (2) discuss the scientific and technical issues associated with developing study endpoints for rare diseases and highlight resources to assist with

addressing these issues, (3) discuss lessons learned from previous PDUFA meeting programs that can be applied to the RDEA Pilot Program, and (4) provide opportunity for public comment on the RDEA Pilot Program and issues associated with rare disease endpoint development.

Meeting sessions will focus on: (1) the RDEA Pilot Program and process, (2) elements of RDEA proposal and meeting packages, (3) addressing issues in developing rare disease endpoints, and (4) experience with other FDA pilot programs. At the end of the public workshop, there will be an opportunity for public comment.

Meeting updates, the agenda, and background materials (if any) will be made available at https://duke.is/5ca3k prior to the workshop.

III. Participating in the Public Workshop

Registration: To register for the public workshop, please visit the following website: https://duke.is/5ca3k. Please provide complete contact information for each attendee, including name, title, affiliation, address, email, and telephone. Registration will end at 11:59 p.m. Eastern Time on June 6, 2023.

Registration is free, and persons interested in attending this public workshop must register to receive a link to the meeting. Registrants will receive a confirmation email after they register.

If you need special accommodations due to a disability, please contact Margolisevents@duke.edu no later than May 5, 2023. Please note, closed captioning will be available automatically.

Requests for Oral Comments: During online registration you may indicate if you wish to speak during a public comment session. We will do our best to accommodate requests to make public comments. Individuals and organizations with common interests are urged to consolidate or coordinate their comment and request time for joint commentary. All requests to make oral comments must be received by 11:59 p.m. Eastern Time on May 26, 2023. FDA will determine

the amount of time allotted to each commenter and the approximate time each comment is to begin and will select and notify participants by June 2, 2023.

Transcript: Please be advised that as soon as a transcript of the public workshop is available, it will be accessible at https://duke.is/5ca3k. The transcript will also be available at https://www.regulations.gov and may be viewed at the Dockets Management Staff (see **ADDRESSES**).

Dated: April 12, 2023.

Lauren K. Roth,

Associate Commissioner for Policy.

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