



DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2021-N-0512]

Considerations for Progressive Multifocal Leukoencephalopathy Clinical Trial Designs; 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 or Agency) is announcing the following public workshop entitled “Considerations for Progressive Multifocal Leukoencephalopathy Clinical Trial Designs.” The purpose of the public workshop is to discuss the challenges and clinical trial design considerations for developing therapeutic products for the treatment of progressive multifocal leukoencephalopathy (PML).

DATES: The public workshop will be held virtually on September 21, 2021, from 10 a.m. to 4:15 p.m., Eastern Time. Submit either electronic or written comments on this public workshop by November 1, 2021. See the SUPPLEMENTARY INFORMATION section for registration date and information.

ADDRESSES: The public workshop will be held in virtual format only.

You may submit comments as follows. Please note that late, untimely filed comments will not be considered. Electronic comments must be submitted on or before November 1, 2021. The <https://www.regulations.gov> electronic filing system will accept comments until 11:59 p.m. Eastern Time at the end of November 1, 2021. Comments received by mail/hand delivery/courier (for written/paper submissions) will be considered timely if they are postmarked or the delivery service acceptance receipt is 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-2021-N-0512 for "Considerations for Progressive Multifocal Leukoencephalopathy Clinical Trial Designs." 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: Lori Benner and/or Antoinette Ziolkowski, Center for Drug Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 22, Rm. 6221, Silver Spring, MD 20993-0002, 301-796-1300.

SUPPLEMENTARY INFORMATION:

I. Background

Progressive Multifocal Leukoencephalopathy (PML) is a rare, often fatal viral disease of the central nervous system that affects patients with immunosuppressive conditions and those treated with immunomodulatory agents. No products are approved for the treatment of PML and no therapeutic development pathway is established for PML. FDA seeks to discuss scientific and regulatory challenges associated with designing clinical trials evaluating PML treatments, and to develop PML clinical trial designs that are feasible, adequate to establish substantial evidence of effectiveness, adequate to characterize the safety profile of investigational treatments, and acceptable to PML patients, clinicians, regulators, and industry.

The Agency encourages healthcare providers, employees of other U.S. Government agencies, academic experts, industry experts, patients and patient advocates, and other stakeholders to attend this public workshop.

II. Topics for Discussion at the Public Workshop

Discussions are planned around the following topics areas:

- Unmet need for PML therapeutics.
- Key trial design considerations, including feasibility, trial populations, selection of control groups, endpoints, adaptive designs, and master protocols.

III. Participating in the Public Workshop

Registration: Persons interested in attending this public workshop must register online by September 20, 2021, midnight Eastern Time using the weblink for this workshop noted in the *Transcripts* section below. Please provide complete contact information for each attendee, including name, title, affiliation, address, email, and telephone.

Requests for Oral Presentations: During online registration, you may indicate if you wish to present during the virtual public comment session and which topic(s) you wish to address. 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 presentations, and request time for a joint presentation, or submit requests for designated representatives to participate in the focused sessions. We will determine the amount of time allotted to each presenter and the approximate time each oral presentation is to begin, and will select and notify participants by September 10, 2021. All requests to make oral presentations must be received by September 3, 2021. If selected for presentation, any presentation materials must be emailed to the ONDPublicMTGSupport@fda.hhs.gov no later than September 16, 2021. No commercial or promotional material will be permitted to be presented or distributed at the public workshop.

Streaming Webcast of the public workshop: This public workshop will be webcast at the following site: <https://collaboration.fda.gov/fdaworkshop092121>

If you have never attended a Connect Pro event before, test your connection at https://collaboration.fda.gov/common/help/en/support/meeting_test.htm. To get a quick overview of the Connect Pro program, visit https://www.adobe.com/go/connectpro_overview. FDA has verified the website addresses in this document, as of the date this document publishes in the *Federal Register*, but websites are subject to change over time.

Transcripts: Please be advised that as soon as a transcript of the public workshop is available, it will be accessible at <https://www.regulations.gov>. It may be viewed at the Dockets Management Staff (see ADDRESSES). A link to the transcript will also be available on the internet at <https://www.fda.gov/drugs/news-events-human-drugs/considerations-progressive-multifocal-leukoencephalopathy-clinical-trial-designs-09212021-09212021>.

Dated: June 15, 2021.

Lauren K. Roth,

Acting Principal Associate Commissioner for Policy.