4164-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No.FDA-2020-N-0837]

Rare Disease Clinical Trial Networks; Request for Information and Comments

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice; request for information and comments.

SUMMARY: The Food and Drug Administration (FDA, the Agency, or we) is announcing the establishment of a docket to obtain information and comments from patients, patient advocates, the scientific community, health professionals, other regulatory and health authorities in the global community, regulated industry, and the general public regarding practical steps and successful approaches to establish a rare disease clinical trials network.

DATES: Submit written or electronic comments and information on the notice by [INSERT DATE 60 DAYS AFTER DATE OF PUBLICATION IN THE *FEDERAL REGISTER*].

ADDRESSES: 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 [INSERT DATE 60 DAYS AFTER DATE OF PUBLICATION IN THE *FEDERAL REGISTER*]. The https://www.regulations.gov electronic filing system will accept comments until 11:59 p.m. Eastern Time at the end of [INSERT DATE 60 DAYS AFTER DATE OF PUBLICATION IN THE *FEDERAL REGISTER*]. 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-2019-N-5464 for " Rare Disease Clinical Trial Networks; Request for Information and Comments." 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.

- 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: Meghana Chalasani, Center for Drug Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 51, Rm. 6304, Silver Spring, MD 20993-0002, 240-402-6525, meghana.chalasani@fda.hhs.gov.

SUPPLEMENTARY INFORMATION:

I. Background

Over the past decade, progress has been made in planning and conducting clinical trials for rare disease drug development. In 2018, for the first time ever, a majority of new molecular entities approved by the FDA were orphan drugs to treat rare diseases. However, of the approximately 7,000 known rare diseases, less than 10 percent have an FDA-approved treatment available. Rare disease drug development continues to be challenged by the small numbers of patients and limited understanding of the variability and progression of each disease.

To support innovation and quality in the drug development pipeline for rare diseases, FDA has proposed establishment of a "Rare Disease Cures Accelerator." The Rare Disease Cures Accelerator would provide a more centralized infrastructure and common platform(s) and approaches to support: (1) rare disease characterization, (2) development of standard core sets of clinical outcome assessments and endpoints relevant to rare conditions, and (3) support conduct of clinical trials in rare disease populations. Following FDA CDER receipt of $10 million in FY 2019 Congressional appropriations for investment and innovation for rare diseases, FDA

launched a set of efforts to begin building capabilities for the first two of these three components. To learn more, please visit FDA's Rare Disease Cures Accelerator Homepage [https://www.fda.gov/drugs/regulatory-science-research-and-education/rare-disease-cures-accelerator].

With this request for information and comments, FDA is interested in understanding what work is currently being done and what work needs to be done to address the third component of its Rare Disease Cures Accelerator--improving the design, conduct, and completion of rare disease clinical trials. FDA is particularly interested in learning practical steps and successful approaches related to startup, implementation, and sustainment of clinical trials networks for rare diseases, including specific considerations for establishing such networks for a range of rare diseases. Because of the small size of rare disease populations and global occurrence of rare conditions, it is considered that the networks needed to support rare disease drug development would also have global reach and operations.

II. Requested Information and Comments

FDA requests input on practical steps and successful approaches to startup, implement, and sustain global clinical trials networks, including specific considerations for establishing such networks for a range of rare diseases. Questions that could be addressed include, but are not limited to, those listed below. It is not necessary to answer all the questions below.

1. What should be the immediate (< 3 years) and long-term objectives of a global clinical trials network?
2. How could a global clinical trials network for rare disease be organizationally structured (e.g., what mix of scientific and clinical disciplines are engaged to staff it; what process or guidance is followed for study protocol design; what standard procedures are

employed for conduct of trials, and related protection of study participants and study data, etc.)? For example:

- Are there experiences that can be shared regarding networks integrating a disease-specific development center with a disease-agnostic operations center?
- Are there experiences that can be shared regarding networks focused on a broad group of rare diseases and collaboration with regional or disease-specific networks?

3. What kind of investigator experience is needed to start up and expand to implement a global clinical trial network (e.g., experience with clinical trial research administration, clinical trial operations, working with pharmaceutical companies in the design, conduct and management of clinical trials)?
4. What are successful models of governance for global clinical trial networks (e.g., role, responsibilities, and composition of various governing bodies)?
5. What are potential opportunities to leverage and/or complement other existing networks (e.g., Institute for Advanced Clinical Trials for Children Network, Duke Clinical Research Institute Pediatric Trial Network, National Institutes of Health (NIH) Rare Diseases Clinical Research Network, NIH Experimental Therapeutics Clinical Trials Network, European Network of Paediatric Research at the European Medicines Agency)?
6. What infrastructure is required to startup, implement, and sustain a global clinical trials network (e.g., required administrative, financial and physical resources, centralized functions, data coordination and network operations, global interoperability)?
7. What level of funding would be needed to establish a network, potentially expand a network, and sustain the network over the long term (e.g., at least 5 years and longer)? A

a range of estimates (e.g., startup costs, annual operating costs) and associated assumptions would be helpful.

8. What are the key milestones and associated timelines for starting up and expanding to implement a global clinical trials network?
9. What are potential challenges or barriers to starting up, implementing, and sustaining a global rare disease clinical trials network?

Dated: May 26, 2020.

Lowell J. Schiller,

Principal Associate Commissioner for Policy.

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