4164-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2019-N-3453]

Promoting Effective Drug Development Programs: Opportunities and Priorities for the Food and Drug Administration's Office of New Drugs; Public Meeting; Request for Comments

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice of public meeting; request for comments.

SUMMARY: The Food and Drug Administration (FDA, the Agency, or we) is holding a public meeting on November 7, 2019 entitled "Promoting Effective Drug Development Programs: Opportunities and Priorities for FDA's Office of New Drugs." The purpose of the public meeting is to solicit specific, actionable policy suggestions that could be implemented in the near-term by the review staff of the Center for Drug Evaluation and Research's (CDER's) Office of New Drugs to promote effective drug development programs without compromising our regulatory standards for the assessment of safety and effectiveness.

DATES: The public meeting will be held on November 7, 2019, from 9 a.m. to 5 p.m. The public meeting may be extended or may end early depending on the level of public participation. Persons can attend the event in-person or via webcast. In-person attendees can also request to give a formal presentation as part of the registration process. See the SUPPLEMENTARY INFORMATION section for registration date and information. Electronic or written comments will be accepted after the public hearing until January 7, 2020.

ADDRESSES: The public meeting will be held at the FDA White Oak Campus, 10903 New Hampshire Ave., Building 31 Conference Center, the Great Room (Rm. 1503), Silver Spring, MD 20993-0002. Entrance for public meeting participants (non-FDA employees) is through Building 1, where routine security check procedures will be performed. For parking and security information, please refer to https://www.fda.gov/AboutFDA/WorkingatFDA/BuildingsandFacilities/WhiteOakCampusInformation/ucm241740.htm.

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 January 7, 2020. The https://www.regulations.gov electronic filing system will accept comments until 11:59 p.m. Eastern Time at the end of January 7, 2020. 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-3453 for "Promoting Effective Drug Development Programs: Opportunities and Priorities for FDA's Office of New Drugs." 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.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.

FOR FURTHER INFORMATION CONTACT: Eithu Lwin, Center for Drug Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 22, Rm. 6236, Silver Spring, MD 20993, 301-796-0728, Eithu.Lwin@fda.hhs.gov.

SUPPLEMENTARY INFORMATION:

I. Background

FDA regulates drugs, including those that are licensed as biological products, under the Federal Food, Drug, and Cosmetic Act (FD&C Act), the Public Health Service Act (PHS Act), and relevant implementing regulations to promote and protect the public health.

The central mission of CDER's Office of New Drugs (OND) is the proper and, where appropriate, flexible implementation of review authorities to ensure that safe and effective drugs and biologics are available to the American people. Rapid scientific and technological advances have enhanced our understanding of disease pathologies and, in many cases, have identified potential actionable targets that translate into drug development programs with an increased emphasis on precision medicine, rare diseases, and defined subsets of common diseases. FDA can help to ensure these innovations reach patients through the development and dissemination of clear policies regarding our expectations and standards.

FDA is engaged in multiple, high-priority policy initiatives to facilitate effective drug development. Effective drug development programs leverage the best available scientific knowledge to characterize the benefits and risks of a potential product and generate the data necessary to support product approval. Current FDA endeavors in this area include evaluating the potential use of real-world evidence in regulatory decision-making and the incorporation of the patient perspective into drug development and review. FDA has previously released comprehensive plans or frameworks for the advancement of these efforts; therefore, these topics are not the focus of this meeting. Instead, stakeholders are encouraged to provide input on these initiatives at topic-specific public meetings or in response to draft guidance.

Consistent with FDA's broader initiatives and modernization efforts, the clinical and scientific leaders of OND are seeking suggestions on where we can provide regulatory clarity to promote innovative and effective drug development across multiple therapeutic areas. FDA will

hold a public meeting on November 7, 2019, from 9 a.m. to 5 p.m, to solicit input from stakeholders regarding where the Office of New Drugs can focus policy priorities while those broader Agency- and Center-wide efforts, described above, continue. In particular, OND welcomes specific policy suggestions that can be implemented in the near-term.

The format of the meeting involves presentations from the public to a panel of leaders from OND review divisions. The Agency will not be inviting specific presenters; rather, with this document, FDA is soliciting presentations from interested stakeholders. FDA also invites interested stakeholders to submit written comments to the docket on the topics described below.

II. Topics for Discussion at the Public Meeting

FDA is soliciting specific, actionable policy suggestions that could be implemented in the near-term by the review staff of CDER's Office of New Drugs to promote effective drug development programs without compromising our regulatory standards for the assessment of safety and effectiveness.

The Agency welcomes any relevant information that stakeholders wish to share at the meeting or in a submission to the docket, but we emphasize that the focus of this meeting is to seek input that is distinct from parallel, topic-specific initiatives related to real-world evidence and patient-focused drug development. Furthermore, to best inform policy priorities, we anticipate that the most informative suggestions would not be specific to a therapeutic area or disease but rather apply across multiple therapeutic areas or diseases. We are particularly interested in the topics that follow.

1. We are interested in input from stakeholders about where OND can provide additional guidance or prioritize additional scientific discussion in the near-term to improve clarity and encourage effective drug development. Given that OND's portfolio includes a

diverse spectrum of drugs and diseases, such input should focus on specific policy needs for various clinical areas linked by a shared therapeutic context (e.g., drugs intended to treat serious, life-threatening rare diseases; non-serious, self-limited conditions; etc.), rather than focusing on any specific disease or condition.

2. Over the past decade, advances in scientific knowledge have led to unprecedented targeting of drugs to the underlying genetic or molecular pathophysiology of a disease. For many diseases, however, the current state of knowledge does not provide opportunities for such precise targeting, but patients living with these diseases require therapeutic innovation as well. Recognizing that each disease has unique considerations, we are interested in specific suggestions for guidance or policy development that OND could undertake to facilitate drug development for diseases not currently amenable to targeted therapies.
3. Some therapeutic areas, particularly those that include serious and life-threatening diseases, have begun to implement novel trial designs, such as the use of master protocols to study multiple therapies and/or multiple diseases under a common infrastructure. We are interested in stakeholders' views regarding the advantages and disadvantages of extending these approaches to additional therapeutic areas, and what guidance development would be most useful.
4. FDA has published many guidances intended to explain the Agency's current thinking regarding drug development topics that are not specific to a particular disease or indication. If stakeholders believe that OND review divisions are implementing these guidances in different ways, which are not explained by case-specific features, this may reflect a need for guidance revision or additional policy development. We are interested

in hearing specific recommendations for topics where further clarity of the Agency's current thinking may be warranted.

5. Innovative approaches can bring additional uncertainty to drug development, since the advantages and disadvantages of the approaches may not yet be fully understood by either the Agency or sponsors because of their novelty. Sometimes, a well-understood development pathway may be chosen solely because of existing precedents in the therapeutic area. We would like to hear how OND can promote effective drug development programs when this tension exists.

III. Participating in the Public Meeting

Registration: Persons interested in attending this public meeting must register online at https://promotingeffectivedrugdevelopmentprograms.eventbrite.com by midnight on October 10, 2019. Please provide complete contact information for each attendee, including name, title, affiliation, address, email, and telephone. Please also indicate whether attendance will be by webcast or in person. Early registration is recommended because seating is limited; therefore, FDA may limit the number of participants from each organization. If registration reaches maximum capacity, FDA will post a notice closing registration at https://www.fda.gov.

If you need special accommodations due to a disability, please contact Eithu Lwin (see FOR FURTHER INFORMATION CONTACT) no later than October 30, 2019.

Requests for Oral Presentations: During online registration you may indicate if you wish to present. To facilitate agenda development, registrants requesting to present will be contacted to provide information regarding which topics they intend to address and the title of their presentation. We will do our best to accommodate requests to present. 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. All requests to make oral presentations must be received by the close of registration on October 10, 2019.

Following the close of registration, we will determine the amount of time allotted to each presenter and the approximate time each oral presentation is to begin, and we will select and notify participants by October 24, 2019. If selected for presentation, registered presenters planning to use an electronic slide deck should submit an electronic copy of their presentation (PowerPoint or PDF), to ONDPublicMTGSupport@fda.hhs.gov with the subject line "Promoting Effective Drug Development Programs: Opportunities and Priorities for FDA's Office of New Drugs" on or before October 31, 2019. If presenters choose not to use a slide deck, they are requested to submit a single slide with their name, affiliation, title of presentation, and contact information. Persons registered to present are encouraged to arrive at the meeting room early and check in at the onsite registration table to confirm their designated presentation time. No commercial or promotional material will be permitted to be presented or distributed at the public meeting.

Streaming Webcast of the Public Meeting: This public meeting will also be webcast. To join the meeting via the Webcast, visit https://collaboration.fda.gov/ond110719/.

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 meeting 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 Agency's website at https://www.fda.gov.

Dated: August 7, 2019.

Lowell J. Schiller,

Principal Associate Commissioner for Policy.

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