Guidelines for Developing a Letter of Intent (LOI) for Externally-led Patient-Focused Drug Development Meetings

Patient-Focused Drug Development (PFDD) aims to more systematically obtain the patient perspective on specific conditions and their treatments. PFDD meetings give FDA and other key stakeholders, including medical product developers, health care providers, and federal partners, an important opportunity to hear directly from patients, their families, caregivers, and patient advocates about the symptoms that matter most to them, the impact the condition has on patients' daily lives, and patients' experiences with currently available treatments.

FDA has conducted over 30 disease-specific PFDD meetings since 2012 and recognizes that there are many more disease areas that can be addressed beyond the PFDD meetings planned and conducted by FDA. To help expand the benefits of FDA's PFDD initiative, FDA welcomes patient organizations to identify and organize patient-focused collaborations to generate public input on these disease areas, and to hold meetings that use FDA-led PFDD meetings as a model. More information about this voluntary program can be found on FDA's Externally-led Patient-Focused Drug Development Meetings webpage.

FDA recommends that patient organizations that are interested in conducting an externally-led PFDD meeting submit a **Letter of Intent (LOI)** that communicates (1) the importance of the meeting in the context of the disease area, and (2) important details regarding the meeting plan. The LOI should be submitted approximately 1 year before the anticipated meeting date. To increase transparency and to provide a single source for interested parties to find information on upcoming EL-PFDD meetings, FDA intends to share the information contained in the LOI, including the name of your organization and a point of contact for further information about the planned meeting, on the FDA website.

FDA's CDER PFDD Staff leads the externally-led PFDD program and will coordinate an internal review of the submitted LOIs with clinical review divisions on a quarterly schedule as follows:

- LOIs submitted December 1 – February 28 will be reviewed starting March 1
- LOIs submitted March 1 – May 31 will be reviewed starting June 1
- LOIs submitted June 1 – August 31 will be reviewed starting September 1
- LOIs submitted September – November 30 will be reviewed starting December 1

After a review and assessment of the LOI on a quarterly basis, FDA's CDER Patient-Focused Drug Development Program Staff will respond to the host organization. For a limited number of meetings that may be of special interest to stakeholders and FDA, the PFDD staff will be

available to provide specific recommendations on the planning of the meeting (e.g., development of agenda, discussion/polling questions), to serve as a resource through monthly check-in calls, and to act as your primary point of contact at FDA. The planning of an externally-led PFDD meeting can be done without being resource intensive. FDA generally does not encourage the use of event planners, consultants, scientific writers, or other external resources, especially when funds may be limited. The key is to begin planning early. FDA encourages patient organizations to consider including externally-led PFDD sessions as part of annual meetings or symposiums to help maximize resources.

Please submit the LOI to patientfocused@fda.hhs.gov.

The LOI should be brief (approximately 3 pages) and communicate the following information:

1. Proposed Disease Area(s), and a discussion of how the proposed disease area(s) fits within the criteria FDA outlined in its PDUFA V PFDD disease area meeting identification process:
    a. Disease area that is chronic, symptomatic, or affects functioning and activities of daily living;
    b. Disease area for which aspects of the disease are not formally captured in clinical trials;
    c. Disease area for which there are currently no therapies or very few therapies, or the available therapies do not directly affect how a patient feels, functions, or survives; and/or
    d. Disease area that has a severe impact on identifiable subpopulations (such as children or elderly).
2. Proposed meeting goals and objectives. Identify the desired key learnings of this effort, and how these learnings may support patient-focused drug development for the disease area(s).
3. The target patient population, characterized by the range in disease or patient characteristics (e.g., severity, years since diagnosis). Discuss any important disease or patient characteristics or experiences that should be reflected (e.g., variations of the disease, a grouping of several diseases, the spectrum of severity, and the spectrum of experiences with current treatments). Describe if you intend to focus on any subpopulations, such as children less than 18 years old, people age 65 and greater, people with metastatic forms of the disease, etc.
4. Proposed meeting date, time, location *(Note: FDA will consider in-person attendance for meetings held in the Washington DC metro area; FDA will consider remote attendance via webcast for meetings held outside the Washington DC metro area.)*
5. Draft outline of the meeting agenda, topic areas, and discussion questions. The meeting agenda should follow the PFDD Meeting Format listed below.
6. Discussion on any other supporting mechanisms to collect patient input (e.g., use of a survey, collecting patient comments, or crowdsourcing methods).
7. Patient outreach and engagement plan. Include a discussion of how you will address patient representation considering patient demographic and disease characteristics.
8. Proposed work products to be generated based on the meeting, (e.g., summary report, webcast, transcript, surveys). Discuss your plan to make this information widely available to the public.
9. Identification of any other collaborators (e.g., other patient groups, financial sponsors, or other key stakeholders) and their role in the meeting. NOTE: The success of an externally-led PFDD meeting will require a joint, aligned effort by multiple patient organizations associated with the disease area, and other interested stakeholders. This effort helps to ensure awareness and increased participation in the meeting by the patient community, enhancing the value of the

meeting as an opportunity to hear from the community. FDA expects multiple patient groups and other stakeholders in a disease space to collaborate in planning, executing, and developing deliverables from the meeting.

10. Identification of any offices that you would like to invite to attend the meeting. For opening remarks, typically a member of the clinical review division who reviews applications related to the condition will be recommended by the division. Your FDA staff liaison (who will be assigned to you after review of the LOI) will work with you early in the planning process to determine an FDA invitee list. The decision about who from the division delivers the opening remarks is at the discretion of the review division director and available staff and resources at the time.
11. Statement of acknowledgement of the following:
    a. FDA's CDER Patient-Focused Drug Development Program Staff will be your liaison and primary point of contact at FDA for your externally-led PFDD meeting. Once your LOI has been reviewed, you will be provided with the contact information of your assigned FDA PFDD Staff liaison.
    b. That the information contained in the LOI (including contact information for the primary point of contact) may be made public and may be posted on the website of FDA prior to the meeting and during the planning stage to ensure transparency of EL-PFDD efforts.
    c. That the information conveyed in the LOI represents the honest and most forthcoming intentions of your organization and that efforts will be made to collaborate with other organizations that represent patients within the disease area. If any of the information above is intentionally misrepresented, the FDA PFDD Staff reserves the right to remove itself, and any FDA staff, from engaging with this externally-led meeting.

Please contact FDA if you have questions. Please note that submission of an LOI does not guarantee that a meeting will occur or that FDA PFDD Staff will participate or attend the meeting.

PFDD Meeting Format

The PFDD meeting format has been developed to ensure that PFDD meetings, including EL-PFDD meetings follow a proven format that focuses the meetings on the information about patients lived experiences that patient groups, medical product developers, and regulators have told FDA are most critical to understand. For a meeting to be considered an EL-PFDD meeting, **the following agenda and questions should be followed exactly**. Any divergence from this model should be discussed with PFDD staff.

Timing: We <u>recommend</u> a half day/4-hour meeting

- **Welcome (5 min)**
- **Opening Remarks (5 min) (FDA Speaker)**
- **Clinical Overview and Background on the condition (10 min)**
- **Overview of Discussion Format (10 min)**
- **Topic 1: Health Effects and Daily Impacts**
  - **Panel (30 min)**
    - A panel of 4-5 patients and/or caregivers provide comments to start the discussion on health effects and daily impacts of the condition.
  - **Large-Group Facilitated Discussion on Topic 1 (65 min)**
    - Patients and patient representatives in the audience are invited to add to the dialogue.
  - **Questions:**
    1. Of all the symptoms that you experience because of your condition, which 1-3 symptoms have the most significant impact on your life? Examples may include chronic pain, constipation, difficulty concentrating, etc.
    2. Are there specific activities that are important to you but that you cannot do at all or as fully as you would like because of your condition? Examples of activities may include sleeping through the night, daily hygiene, participation in sports or social activities, intimacy with a spouse or partner, etc.
    3. As it relates to your condition, what does a good day look like? What does a bad day look like?
    4. How have your condition and its symptoms changed over time?
       a. Would you define your condition today as being well managed?
    5. What worries you most about your condition?
- Break (30 min)
- **Topic 2: Current Approaches to Treatment**
  - **Panel (30 min)**
    - A panel of 4-5 patients and/or caregivers will provide comments to start the discussion on current approaches to treatment for the condition.
  - **Large-Group Facilitated Discussion on Topic 2 (65 min)**
    - Patients or patient representatives in the audience are invited to add to the dialogue.
  - **Questions:**
    1. What are you currently doing to help treat your condition or its symptoms? Examples may include prescription medicines, over-the-counter products, and other therapies including non-drug therapies such as diet modification.
    2. How has your treatment regimen changed over time, and why?
    3. How well does your current treatment regimen treat the most significant symptoms of your disease? For example, how well do your treatments improve your ability to do specific activities?
       a. How well have these treatments worked for you as your condition has changed over time?

4. What are the most significant downsides to your current treatments, and how do they affect your daily life? Examples of downsides may include going to the hospital or clinic for treatment, time devoted to treatment, restrictions on driving, etc.
5. Short of a complete cure, what specific things would you look for in an ideal treatment for your condition?
    a. What would you consider to be a meaningful improvement (for example symptom improvements or functional improvements) in your condition that a treatment could provide?

- **Closing Remarks (5 min)**