

## 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. The patient perspective is critical to help provide context when FDA makes regulatory decisions for new drugs. 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. This input can inform FDA's decisions and oversight both during drug development and during our review of a marketing application.

FDA has conducted over 29 disease-specific PFDD meetings since 2012. FDA 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. 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.

After a review and assessment of the LOI, 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 resources 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](mailto: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.
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 specific FDA staff that you would like to invite to attend or provide remarks during the meeting. If you are not certain who the appropriate FDA staff may be, 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.
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.
12. 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.