

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

The [Patient-Focused Drug Development](#) (PFDD) initiative aims to more systematically obtain the patient perspective on specific diseases and their treatments. The patient perspective is critical in helping FDA understand the context in which regulatory decisions are made for new drugs. PFDD meetings give FDA and other key stakeholders, including medical product developers, health care providers, 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 disease 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 25 disease-specific PFDD meetings to more systematically obtain the patient perspective on specific diseases and their treatments. FDA recognizes that there are many more disease areas that can be addressed beyond the PFDD meetings planned and conducted by the 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 other disease areas, using the process established by FDA-led PFDD meetings as a model. More information can be found on FDA's [Externally-led Patient-Focused Drug Development Meetings](#) webpage.

FDA recommends that patient organizations who 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.

Please submit the letter of intent to patientfocused@fda.hhs.gov. FDA's CDER [Patient-Focused Drug Development Program Staff](#) will receive and review the letter.

The letter of intent (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;
 - d. Disease area that have 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 particular 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 (e.g., 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 of 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 of the meeting, (e.g., summary report, webcast, transcript, surveys). Discuss your plan to make this information more 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.
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 LOI) will work with you early in the planning process to determine an FDA invitee list.
11. Statement of acknowledgement that FDA's CDER [Patient-Focused Drug Development Program Staff](#) will be your liaison and primary point of contact at the 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 staff liaison.