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

As part of its commitments under the Prescription Drug User Fee Act reauthorization of 2012, FDA has taken several steps to inform the benefit-risk assessments that inform CDER's regulatory decisions concerning new drugs. Among these efforts is the Patient-Focused Drug Development initiative (PFDD) that aims to more systematically obtain the patient perspective on specific diseases and their currently available treatments. Through PFDD, FDA is convening a series of at least 20 public meetings during FY 2013-2017 focused on specific disease areas.

The Agency recognizes that there are many more disease areas than can be addressed in the planned FDA meetings under PDUFA V. We welcome patient organizations to identify and organize externally-led PFDD meeting to generate public input on other disease areas, using the process established through the PDUFA V Patient-Focused Drug Development as a model. FDA will determine its level of participation in these meetings on an individual basis, taking into account a number of factors, including any identified need for a better understanding of patient perspective, recent interactions with patient stakeholders, proposed meeting details, and FDA staff capacity. More information can be found here: Externally-led Patient-Focused Drug Development Meetings

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 Office of Strategic Programs will receive and review the letter.

The letter of intent (LOI) should be brief (recommended approximately 5 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 that FDA participation is more feasible if the meeting is held in the Washington DC metro area)*
5. Planned meeting format (e.g., in-person and/or web participation; patient testimonies and/or facilitated discussion). Include a 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 , including a summary report, webcast, transcript, surveys, etc. 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.