U.S. FOOD & DRUG ADMINISTRATION

FREQUENTLY ASKED QUESTIONS (FAQS)

FDA's condition-specific meeting reports webpage provides links to certain publicly available external reports and resources relating to patient experience data. This webpage hosts an alphabetical listing of **publicly available** condition-specific meeting reports and other information related to patients' experience. These meetings include FDA-led Patient-Focused Drug Development (PFDD) meetings, Externally-led PFDD meetings, and Patient Listening Sessions. Other information includes proposed draft guidance relating to patient experience data, natural history studies, or other condition-specific background on condition and discussion of unmet medical need. The patient community, patient advocates, researchers, drug developers, clinicians, and federal agencies may find these materials useful.

What is patient experience data[1]?

Under Title III, Section 3001 of the 21st Century Cures Act of 2016, "patient experience data" are data that are collected by any persons and are intended to provide information about patients' experiences with a disease or condition. Patient experience data can be interpreted as information that captures patients' experiences, perspectives, needs, and priorities related to (but not limited to): 1) the symptoms of their condition and its natural history; 2) the impact of the conditions on their functioning and quality of life; 3) their experience with treatments; 4) input on which outcomes are important to them; 5) patient preferences for outcomes and treatments; and 6) the relative importance of any issue as defined by patients.

Who can provide a publicly available website link to a report or other resource?

FDA welcomes patients, families and caregivers of patients, patient advocacy organizations, disease research foundations, academic and medical researchers, expert practitioners, drug developers, and other interested persons to submit website links to publicly-available reports (e.g., meeting reports) and other resources related to or containing patient experience data on the topics outlined below. Submissions may be submitted by an individual, organization, or collaboration group that is identified as an author.

What types of resources may be included?

FDA intends to post website links to reports and other resources that fall within the following categories:

- Externally-led Patient-Focused Drug Development (PFDD) or Other Stakeholder Meeting Reports: To help expand the benefits of FDA's PFDD program, FDA welcomes patient organizations to identify and organize patient-focused collaborations to generate public input on other disease areas. Submitted links to summary meeting reports from these externally-led PFDD meetings may be posted on the webpage. FDA also welcomes submission of meeting reports from other stakeholder meetings collecting patient perspectives on disease burden and treatment burden.

- Proposed Draft Guidance Relating to Patient Experience Data: Final guidance documents issued by FDA represent the Agency's current thinking on a particular subject, for example, the design of certain clinical trials. The FDA issues draft guidances to receive public comment as it considers whether to adopt final guidance.

  External stakeholders may submit[2] website links to publicly-available *proposed* draft guidances they have drafted relating to patient experience data, and which they propose that the FDA adopt, as a resource for the medical product development community on the *Condition-Specific Meeting Reports and Other Information Related to Patients' Experience* webpage. As with the other resources on the website, the proposed draft guidances and their content are not endorsed by FDA. Additional FDA guidance on

[1] https://www.fda.gov/downloads/Drugs/NewsEvents/UCM586200.pdf

[2] External stakeholders may also submit proposed draft guidances following the procedures for submission of external guidances provided in 21 CFR 10.115(f)(3).

developing and submitting proposed draft guidance relating to patient experience data for consideration by FDA is under development.

Proposed draft guidance relating to patient experience data may include (but is not limited to) documents that address:

  - clinical trial considerations (e.g., planning, conduct, patient enrollment and participation)
  - considerations for the needs of a patient subpopulation (e.g., a pediatric, elderly, or a subpopulation with particular co-morbidities)
  - disease-specific endpoints that matter to patients that may be considered for inclusion in clinical development programs in that disease area

- Natural History Studies or other Disease-specific Background on Condition and Discussion of Unmet Medical Need: Natural history studies track the course of disease over time, identifying demographic, genetic, environmental, and other variables that correlate with its development and outcomes in the absence of treatment. Natural history studies include retrospective studies, prospective studies, and survey studies. If these studies are published on a public website, external stakeholders may submit the website link. The posted study report should adhere to good research practices and sufficiently describe the study's methods, protocols, and informed consent procedures. Website links to other publicly-available reports or documents providing disease-specific background on the condition and unmet medical need may also be submitted.

What types of resources may not be included?

Clinical studies of marketed or investigational therapies will not be published on this webpage. Reports or resources that FDA considers to be directly related to a product application (e.g., a patient preference study that involves a product under review) will not be published on this webpage. Individual patient narratives or testimonies are considered beyond the scope of this webpage.

How can you submit a publicly available website link to FDA?

To provide FDA with a publicly-available website link to your report or resource, please email PFDDresources@fda.hhs.gov. If your submission meets the guidelines outlined on the webpage, in this FAQ document, and in the Cover Page Guidelines, FDA generally intends to host the submitted link on the webpage. We request that you include a cover page or similar opening statement as part your report or resource to provide information about the study authors, support for the study, and related information. The cover page or statement may be included within the report or resource itself, or it may be hosted as a separate document on the same website link as the report or resource. Please review the cover page guidelines here.

FDA will not review any reports or resources prior to submission in order to help the submitter prepare or revise the report or resource for posting. By posting links to these resources, FDA does not endorse those external resources, their content, sponsors, or authors. Submitters of linked reports and resources should refrain from making any statement that indicates, implies, or suggests that FDA has endorsed the external resource by hosting your link, including by use of the FDA logo.

FDA's CDER Patient-Focused Drug Development Staff is the main point of contact for this webpage. If you have any questions, please email us at PFDDresources@fda.hhs.gov.

What if you've previously shared a report or resource with FDA prior to the launch of this website, and you don't see it on this webpage?

FDA has received many patient-focused reports and resources over the years. As we continue to develop this webpage, we would appreciate your help in this effort! If you do not see your report or resource on the webpage, please resubmit your report or resource to FDA, using the process outlined above.