

Methodologic and Other Challenges Related to Patient Experience Data:

Priorities for Future Work

September 30, 2026

U.S. Food and Drug Administration

Methodologic and Other Challenges Related to Patient Experience Data: Priorities for Future Work

Under the seventh iteration of the Prescription Drug User Fee Act (PDUFA VII), incorporated as part of the FDA User Fee Authorization Act of 2022, FDA committed to facilitate the advancement and use of systematic approaches to collect and utilize robust and meaningful patient and caregiver input that can more consistently inform drug development and, as appropriate, regulatory decision-making. This included issuing a [Request for Information \(RFI\)](#) to elicit public input on methodologic challenges related to patient experience data, and other areas of greatest interest or concern to public stakeholders. The RFI was published on May 2, 2023, and the public comment period was open until July 3, 2023. A [summary of the comments](#) was published on December 12, 2023. The summary highlighted key themes from stakeholders, including requests for greater clarity on how FDA utilizes patient experience data for regulatory decision-making, clarification on concepts and tools outlined in the Patient-Focused Drug Development (PFDD) Guidance Series, pathways for efficient communication with FDA about patient experience data, methods to improve patient involvement and obtain representative input, and expectations regarding submissions of patient experience data.

The input received in response to the RFI helped inform the topics for two public workshops that were held on December 13, 2024, and September 18-19, 2025. The [2024 workshop](#) provided an overview of the different types of patient experience data, with examples of how patient experience data has informed reviews of marketing applications. The workshop discussed considerations for submitting patient experience data to FDA as part of a marketing application, including what information should be in the Reviewers Guide and experiences related to the submission of patient experience data. In addition, there were presentations and panel discussions on selected methodologies and the challenges and opportunities they present, specifically addressing Delphi methods, embedded qualitative interviews, and the use of age-normed scores and repurposed clinical outcome assessments (COAs).

As a follow-up to the 2024 workshop, the [2025 workshop](#) featured presentations and panel discussions on selected methodologies and related topics, specifically addressing meaningful aspect(s) of health and concept of interest, patient baseline symptom severity, and meaningful score regions. The workshop also featured the presentation of a draft updated COA Evidence Dossier Template, developed in response to longstanding stakeholder requests for modernized submission guidance building on the appendix in the 2009 Guidance for Industry, *Patient-Reported Outcome Measures: Use in Medical Product*

Development to Support Labeling Claims. The template is designed to facilitate the submission of evidence to FDA in support of Clinical Outcome Assessments, consistent with the PFDD Guidance Series. Additionally, the workshop explored the intersection of data standardization and statistical programming and highlighted how standardized data submission formats and reproducible code can support efficient regulatory review.

These workshops, together with the input received in response to the RFI and discussions during public engagements, have helped FDA identify the following priorities for future work:

Training and Outreach

Supplemented by the use of the Intergovernmental Personnel Act, grants, and contracts, FDA will expand internal staff training and external outreach to industry sponsors and other involved stakeholders with an emphasis on PFDD methods and tools-related guidance to achieve broad acceptance and integration into regulatory decision-making across review divisions and industry development programs. FDA will continue to support the development of [educational modules](#) to train FDA staff, industry, academic researchers, and patient groups about the PFDD guidance series. With respect to internal staff training, after piloting in-service trainings, FDA will expand these trainings to additional review divisions. For external outreach, FDA will continue to conduct targeted outreach to industry and methodological consulting companies to provide a variety of presentations, sessions, and resources. Building on these efforts, FDA will support the development and publication of additional manuscripts to further explain PFDD and related guidance concepts.

Finalizing Guidance Documents

FDA will finalize two draft guidances intended to facilitate the advancement and use of systematic approaches to collect and use robust and meaningful patient and caregiver input to inform medical product development. FDA will finalize the draft guidance for industry entitled [ICH E22 General Considerations for Patient Preference Studies](#). When finalized, this guidance will provide general principles for the use, design, conduct, analysis, and submission of patient preference studies aimed at informing drug development, regulatory submission and evaluation, drug approvals, and maintenance of such approvals. FDA will also finalize the draft guidance for industry, FDA staff, and other stakeholders entitled [Patient-Focused Drug Development: Incorporating Clinical Outcome Assessments into Endpoints for Regulatory Decision-Making](#). When finalized, this guidance will provide methods, standards, and technologies for collecting and analyzing COA data

for regulatory decision-making, including selecting the COA-based endpoint and evaluating the meaningfulness of the treatment effect on the endpoint. In addition, FDA will update the [Patient-Focused Drug Development Glossary](#) to incorporate key terms defined in these guidances.

Evidence Dossier Template

Based on the comments received through the public docket for the 2025 workshop, FDA will work to finalize the draft evidence dossier template. This template will provide sponsors with a recommended format for organizing COA information and clarify what supporting data is recommended to support that a COA is fit-for-purpose, while maintaining flexibility for sponsors to adapt how they present this information. This will improve review efficiency, promote consistency across submissions, and enhance communication by reducing the number of information requests and facilitating discussions early on between sponsors and FDA. When finalized, this template will replace the Appendix titled, *Information on a PRO Reviewed by the FDA in the 2009 Guidance for Industry, Patient-Reported Outcome Measures: Use in Medical Product Development to Support Labeling Claims*.

Submitting Patient Experience Data and Proprietary COA Supporting Information

Informed by panel discussions held during the workshops, FDA will continue to encourage and engage in conversations with industry about purposefully submitting patient experience data as part of meeting packages and marketing applications. This also includes encouraging the use of tools like the Reviewers Guide and Patient Experience Data Table to facilitate efficient review of patient experience data, and reinforcing expectations for clearly articulating the rationale and supporting evidence, indicating what data is being submitted and where it is located, and communicating how sponsors intend FDA to use the data. A recognized challenge in the review of patient experience data is that many COAs used in drug development are proprietary instruments whose full supporting documentation, including validation studies, normative data, and scoring algorithms, cannot be included directly in a sponsor's marketing application without disclosing copyright-protected information. Drug Master Files (DMFs) are a potential mechanism by which proprietary COA developers could submit confidential supporting documentation to FDA, enabling reviewers to access instrument-level evidence when evaluating a COA's use in a regulatory submission. FDA will include this as part of ongoing discussions regarding efficient review of patient experience data submissions.

PFDD Meetings

FDA will continue to support PFDD meetings to systematically obtain the patient perspective on specific diseases and their treatments. FDA issued an RFI in May 2026 to collect examples of how previous PFDD meetings have impacted stakeholders' drug development efforts. This includes impacts on community engagement, research priorities, advocacy strategies, medical product development programs, clinical practice, and other areas of interest. The input received in response to this RFI has helped stakeholders, including FDA, better understand the broad impact of these meetings. This understanding reinforces FDA's commitment to continue to support the PFDD meeting program.

Continuing Collaboration Across FDA

PFDD work is not done in a silo. PFDD concepts, tools, and staff are leveraged by many activities across FDA such as

- FDA's Rare Disease Innovation Hub ([the Hub](#))
- [Digital Health Technologies](#) (DHTs), [Digitally Derived Measures](#), and [Digitally Derived Endpoints](#)
- Artificial intelligence ([AI](#)) and machine learning
- Rare Disease Endpoint Advancement ([RDEA](#)) Pilot Program
- Drug Development Tools ([DDT](#)) Qualification Program
- CDER's Accelerating Rare Disease Cures ([ARC](#)) Program
- CDER's Center for Clinical Trial Innovation ([C3TI](#)).

Patient and caregiver perspectives can be vital for trial innovations to be successfully implemented. Coordinated efforts ground current and future programs in achieving what matters most to patients and caregivers. FDA staff will continue to work across programs and educational efforts consistent with the goals of the PFDD program.

Acknowledgment

FDA is grateful for the participation of patients, caregivers, medical product developers, academic researchers, and other stakeholders in the workshops and for their input in addressing methodologic and other challenges related to patient experience data. This input will continue to inform efforts to advance and use systematic approaches that collect and utilize meaningful patient and caregiver input to inform drug development and, as appropriate, regulatory decision-making.