NATIONAL HEALTH COUNCIL

June 30, 2026

Dockets Management Staff (HFA-305)
Food and Drug Administration
5630 Fishers Lane, Rm. 1061
Rockville, MD 20852

RE: Impacts of Patient-Focused Drug Development Meetings; Establishment of a Public Docket; Request for Information and Comments [FDA-2026-N-3947]

Submitted electronically via regulations.gov

The National Health Council (NHC) appreciates the opportunity to provide comments on the Food and Drug Administration's (FDA's) request for information (RFI) on the impacts of Patient-Focused Drug Development (PFDD) meetings. FDA's efforts are consistent with the NHC's view that policy cannot be considered patient-centered unless it meaningfully improves the real-world experiences of patients and caregivers. The comments below focus on how FDA can make the impact of PFDD meetings more visible, interpretable, and useful without imposing unnecessary burden on patient communities. The NHC also encourages FDA to use this effort to reinforce that PFDD is most effective when patient input results in concrete follow-on actions.

Created by and for patient organizations more than 100 years ago, the NHC convenes organizations from across the health ecosystem to forge consensus and drive patient-centered health policy. We promote increased access to affordable, high-value, comprehensive, accessible, and sustainable health care. Made up of nearly 200 national health-related organizations and businesses, the NHC's core membership includes the nation's leading patient organizations. Other members include health-related associations and nonprofit organizations including the provider, research, and family caregiver communities; and businesses and organizations representing biopharmaceuticals, devices, diagnostics, generics, and payers.

The NHC recognizes FDA's continued focus on PFDD as part of a broader effort to ensure that medical product development and regulatory decision-making are informed by the experiences, needs, priorities, and preferences of patients and caregivers. FDA-led PFDD and externally led PFDD (EL-PFDD) meetings have helped create a structured way for patients, caregivers, family members, patient advocates, clinicians, researchers, and medical product developers to discuss the aspects of disease and treatment that matter most to affected communities. By creating a structured forum for these perspectives, PFDD meetings can help incorporate patient experience information into the decisions that affect how therapies are studied, reviewed, and communicated. The comments below therefore emphasize both the value of PFDD meetings and the need for a practical framework for applying their outputs.

The NHC views FDA's effort to examine the impact of PFDD meetings as constructive and timely, particularly given the significant time, expertise, and organizational capacity the patient community has invested in these activities. Assessing the value of these meetings should extend beyond the number of meetings held, attendees reached, or reports published to include whether their outcomes lead to concrete action. Successful outcomes may take many forms, including helping patient communities articulate priorities, inform research questions, support more patient-centered clinical development, strengthen patient-facing materials, improve clinical understanding, and make patient experience data more visible and usable across the medical product lifecycle.

At the same time, the NHC encourages FDA to remain attentive to the practical limitations of any retrospective impact assessment. Many important effects of PFDD meetings are not captured in public regulatory documents or attributed explicitly to a specific meeting. Some emerge within patient communities, where meetings help create shared language, identify unmet needs, support advocacy strategies, and inform future engagement. Others develop through relationships among patient organizations, academic researchers, clinicians, and medical product developers. Downstream impacts may extend to endpoint development, clinical outcome assessment work, trial feasibility, patient education, clinician education, benefit-risk assessment, or post-market evidence planning in ways that are difficult to trace through a single public document.

For that reason, the NHC recommends that FDA use this docket to collect examples of PFDD meeting impact and also to identify how future PFDD outputs can be documented, connected to follow-on evidence generation, and made more useful for patients, caregivers, patient organizations, researchers, sponsors, clinicians, and FDA review staff. The value of this effort will depend on whether FDA receives examples and then appropriately applies them to develop a consistent and usable framework for patient-centered evidence across the lifecycle. Examples alone will have limited value unless stakeholders can understand how to act on them. The recommendations below are intended to support that practical orientation.

Summary of Recommendations

The NHC supports FDA's effort to better understand the effects of PFDD meetings and recommends that the Agency use the information collected through this docket to make PFDD outputs more visible, useful, and connected to patient-centered evidence generation in practice. These recommendations are intended to help FDA translate the examples submitted to this docket into practical tools that can be used by patient organizations, researchers, sponsors, clinicians, and review staff. They are also intended to preserve methodological flexibility, recognizing that patient communities vary substantially in size, resources, infrastructure, and evidence needs. The NHC encourages FDA to consider these recommendations as interconnected and mutually reinforcing rather than as isolated steps.

With that framing, the NHC specifically recommends that FDA:

- Synthesize the information submitted to this docket into a public PFDD impact framework that describes how PFDD meetings can affect patient-community engagement, research priorities, medical product development, regulatory decision-making, clinical practice, and patient-community capacity.
- Develop practical case examples showing how PFDD meeting input has been used across the medical product lifecycle, including endpoint development, clinical trial design, patient preference research, benefit-risk assessment, patient-facing materials, post-market evidence generation, and clinician or patient education.
- Clarify that PFDD meetings are valuable but are one method within a broader patient-engagement and evidence-generation continuum, and that meeting outputs may need to be supplemented by additional fit-for-purpose evidence depending on the intended use.
- Encourage voluntary, standardized-but-flexible approaches to documenting PFDD meeting outputs and downstream impacts, including an optional template that patient organizations and other stakeholders can use without creating unnecessary administrative burden or creating new regulatory submission expectations.
- Promote transparency regarding meeting objectives, participant characteristics, recruitment strategies, known limitations, and plans for follow-up engagement with populations that may not have been reached adequately.
- Support lower-resource patient organizations by identifying practical approaches to documenting patient experience and PFDD impact when a large EL-PFDD meeting or extensive follow-on research program is not feasible.
- Improve access to PFDD meeting reports, Voice of the Patient reports, impact summaries, and related resources through a searchable repository or compendium that helps stakeholders learn from prior work and avoid unnecessary duplication, while focusing on public, voluntary, and non-confidential materials.
- Continue engaging patients, caregivers, patient organizations, researchers, sponsors, clinicians, and other stakeholders as FDA determines how to communicate and apply the lessons from this docket.

The NHC emphasizes that any FDA framework, template, repository, or set of case examples should be voluntary, illustrative, and non-prescriptive. These tools should help stakeholders understand possible pathways for using PFDD meeting outputs but should not create new regulatory submission requirements, expectations that every development program must conduct or rely on a PFDD meeting, or pressure to disclose confidential or proprietary development information. This guardrail is important to preserve flexibility, encourage continued collaboration, and ensure that PFDD infrastructure supports patient-centered evidence generation without becoming a new administrative exercise.

NHC's Longstanding Support for Patient-Centered Evidence Across Medical Product Development

The NHC has long supported FDA's efforts to advance patient-focused drug development and to strengthen the role of patient experience data in medical product

development and regulatory decision-making. Across prior comments on FDA's PFDD guidance series and related patient-centered evidence initiatives, the NHC has encouraged FDA to continue developing a framework that treats patient input as a meaningful source of evidence, supports appropriate methodological rigor, and recognizes the many points across the medical product lifecycle where patient perspectives can improve the relevance, feasibility, and interpretability of development programs. The NHC has also emphasized that patient engagement should not be understood only as a mechanism for collecting information from patients, but as a process through which patients, caregivers, and patient organizations can help shape research questions, identify meaningful outcomes, improve study design, and support the development of evidence that reflects patients' lived experience.[1,2,3]

PFDD meetings are one of the clearest examples of how patient engagement can produce value beyond a single regulatory interaction, but their greatest value is realized when meeting outputs are connected to the broader PFDD and patient-centered evidence continuum. In prior comments, the NHC has encouraged FDA to make clear that PFDD is relevant across all stages of development and should not be construed as limited to clinical trials or to the development of clinical outcome assessments. The NHC continues to believe that patient input can inform early research priorities, target product profiles, endpoint selection, clinical outcome assessment development or modification, patient preference research, benefit-risk assessment, patient-facing materials, trial feasibility, recruitment and retention strategies, post-market evidence generation, and communication with patients and clinicians. From the patient perspective, the value of PFDD meetings depends not only on whether a meeting is held or a report is produced, but also on whether the information gathered is translated into decisions, tools, evidence-generation plans, and development strategies that are more responsive to what patients identify as meaningful.[4,5]

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[1] National Health Council, "NHC Comment Letter on FDA PFDD Guidance 2 and 3 [Draft Guidance on Patient-Focused Drug Development: Collecting Comprehensive and Representative Input]," September 11, 2018, https://nationalhealthcouncil.org/wp-content/uploads/2019/12/NHC%20Comment%20Letter%20on%20FDA%20PFDD%20Guidance%202%20and%203.pdf;

[2] National Health Council, "Overview of Final Patient-Focused Drug Development Guidance 1," April 22, 2020, https://nationalhealthcouncil.org/blog/overview-of-final-patient-focused-drug-development-pfdd-guidance-1/;

[3] National Health Council, "Elevating and Improving Patient-Focused Drug Development," May 19, 2020, https://nationalhealthcouncil.org/blog/blog-elevating-and-improving-patient-focused-drug-development/.

[4] National Health Council, "NHC Comments on Patient-Focused Drug Development Guidance: Methods To Identify What Is Important to Patients and Select, Develop, or Modify Fit-for-Purpose Clinical Outcome Assessments," December 14, 2018, https://nationalhealthcouncil.org/wp-content/uploads/2019/12/NHC-Comments_PFDD_2_draft_guidance.pdf

[5] National Health Council, "NHC Comments on PFDD Guidance 4," February 3, 2020, https://nationalhealthcouncil.org/letters-comments/nhc-submits-comments-on-fda-pfdd-guidance-4/.

The NHC has also consistently encouraged FDA to support a flexible, fit-for-purpose approach to patient engagement methods. PFDD meetings can help identify themes, surface concepts of interest, document areas of unmet need, and establish a shared understanding of disease and treatment burden. At the same time, they are not a substitute for every other form of patient-centered evidence generation. Depending on the intended use, PFDD meeting outputs may need to be supplemented by additional qualitative research, quantitative studies, patient preference studies, natural history studies, registries, social media listening, advisory groups, or other methods designed to address specific research questions. The NHC therefore recommends that FDA describe PFDD meetings as part of a broader evidence-generation continuum, rather than as a singular or isolated patient-engagement activity.[6,7,8]

Representativeness is also important in considering how PFDD meeting impacts should be documented and understood. The NHC's prior work emphasizes that patient input does not need to be statistically representative in every context to be valuable. However, stakeholders should be transparent about the objectives of engagement, the populations reached, the populations not reached, and the implications of those limitations for the intended use of the information. In the context of PFDD meetings, reports and impact summaries should describe who participated, how participants were identified or recruited, what follow-up engagement may be needed, and how meeting outputs are being interpreted or supplemented. This transparency would help FDA, patient organizations, researchers, and medical product developers better understand when PFDD meeting outputs are sufficient for a given purpose and when additional evidence generation is warranted.[9,10]

The NHC has also consistently encouraged FDA to provide practical examples that help stakeholders understand how patient input can be used in development and regulatory contexts. That need remains central to the current RFI. FDA can strengthen the field by identifying examples of how PFDD meeting outputs have informed research agendas, endpoint development, clinical trial design, patient preference studies, benefit-risk assessment, patient-facing materials, clinical education, shared decision-making tools, and post-market evidence activities. These examples should include direct regulatory uses where available, but they should also include indirect and upstream uses that may be less visible to FDA unless stakeholders are asked to describe them. In many cases, the most important impacts of PFDD meetings may occur before a formal submission is

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[6] National Health Council, "NHC Comment Letter on FDA PFDD Guidance 2 and 3."

[7] National Health Council, "NHC Comments on Patient-Focused Drug Development Guidance."

[8] National Health Council, "NHC Comments on PFDD Guidance 4."

[9] National Health Council, "NHC Comments on PFDD Guidance 2," December 18, 2019, https://nationalhealthcouncil.org/letters-comments/nhc-comments-on-pfdd-guidance-2/

[10] National Health Council, "NHC Comments on Patient-Focused Drug Development Guidance."

made, after a product is approved, or in patient community and research settings that are not routinely captured through FDA's existing processes.[11,12,13]

The NHC therefore encourages FDA to use this RFI as an opportunity to connect its PFDD guidance with a more practical and visible account of how PFDD meetings are being used. Doing so would help patient organizations, sponsors, researchers, clinicians, and regulators move from general support for patient engagement to a clearer understanding of what effective use of patient input looks like in practice. It would also reinforce that patient-centered evidence should be integrated in a manner that is scientifically sound, transparent, appropriately contextualized, and responsive to the needs and capacity of patient communities.[14,15,16,17]

Strengthening Patient-Community Engagement and Shared Understanding of Disease Burden

PFDD meetings have helped patient communities identify, organize, and communicate shared priorities. For many conditions, especially rare diseases, pediatric conditions, chronic conditions with heterogeneous manifestations, and conditions in which symptoms or treatment burdens may be underrecognized, a PFDD meeting may be the first opportunity for a broad set of affected individuals and stakeholders to discuss the patient experience in a public and organized way. The value of these meetings extends beyond information collection to include the shared understanding that can emerge when stakeholders engage with patient and caregiver experiences in a structured setting.

This aspect of PFDD meetings is particularly important areas where the published literature or traditional endpoints do not capture the daily realities patients describe. Many patients and caregivers live with symptoms, daily-life limitations, treatment burdens, and uncertainty that may not be fully reflected in clinical literature, traditional endpoints, or routine clinical encounters. A PFDD meeting can help make those experiences visible and can give patient organizations, researchers, sponsors, clinicians, and FDA a more complete understanding of what affected individuals consider most important. When these perspectives are not captured, development

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[11] National Health Council, Tackling Representativeness: A Roadmap and Rubric (Washington, DC: National Health Council, 2017), https://nationalhealthcouncil.org/wp-content/uploads/2019/12/Representativeness-in-Patient-Engagement.pdf

[12] National Health Council, "NHC Comment Letter on FDA PFDD Guidance 2 and 3."

[13] National Health Council, "NHC Comments on Patient-Focused Drug Development Guidance."

[14] National Health Council, "NHC Comment Letter on FDA PFDD Guidance 2 and 3."

[15] National Health Council, "NHC Comments on Patient-Focused Drug Development Guidance."

[16] National Health Council, "NHC Comments on PFDD Guidance 2."

[17] National Health Council, "NHC Comments on PFDD Guidance 4."

programs may overlook outcomes or burdens that are central to patients' assessments of a therapy's real-world value.

Public examples suggest that PFDD meetings have been used to gather patient and caregiver insights about daily life, most challenging symptoms, treatment limitations, desired treatment attributes, and outcomes that represent changes patients consider important or beneficial. In several cases, meetings were supplemented by surveys, written comments, listening activities, or other outreach intended to broaden participation and support more durable Voice of the Patient resources. This pattern reinforces that PFDD meetings are often only one part of a larger engagement process. These supplemental activities can help broaden the evidence base while preserving the meeting's role as a focal point for community discussion.[18,19,20,21,22,23,24,25]

PFDD meetings can also help patient organizations refine their own engagement strategies by revealing which patient subgroups are being reached effectively, where additional outreach is needed, how caregiver perspectives should be incorporated, and which questions require additional research. For patient organizations with limited resources, the process of organizing or participating in a PFDD meeting may also build capacity in outreach, facilitation, evidence generation, dissemination, and stakeholder engagement. Experience gained through the process may also help organizations determine whether they need additional outreach to caregivers, people with advanced disease, individuals in rural areas, or other groups whose perspectives may not be well represented in initial activities.

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[18] Arthritis Foundation, "What's Important to Juvenile Idiopathic Arthritis Patients and Families," accessed June 16, 2026, https://www.arthritis.org/news/whats-important-juvenile-idiopathic-arthritis-jia.

[19] Lupus Foundation of America, "Patient-Focused Drug Development Meeting," accessed June 16, 2026, https://www.lupus.org/PFDD-meeting.

[20] Celiac Disease Foundation, "CDF Presents Patient-Focused Drug Development Survey Data to FDA," April 28, 2015, https://celiac.org/2015/04/28/cdf-presents-patient-focused-celiac-drug-development-survey-data-fda/.

[21] Asthma and Allergy Foundation of America, *Voice of the Patient Report on Asthma in Childhood* (Landover, MD: Asthma and Allergy Foundation of America, 2024), https://aafa.org/wp-content/uploads/2024/08/Voice-of-the-Patient-Report-on-Asthma-in-Childhood.pdf.

[22] Arthritis Foundation, *Osteoarthritis Voice of the Patient Report* (Atlanta: Arthritis Foundation, 2017), https://www.arthritis.org/getmedia/6f33fa0d-afed-4800-9238-056460c37ae2/OA-Voice-of-the-Patient-Report.pdf.

[23] Arthritis Foundation, *Juvenile Idiopathic Arthritis Voice of the Patient* (Atlanta: Arthritis Foundation, 2020), https://www.arthritis.org/science/events-publications/jia-vop.

[24] Asthma and Allergy Foundation of America, *Voice of the Patient Report on Asthma in Childhood*.

[25] Cure SMA, "PFDD Meeting Summary," accessed June 16, 2026, https://www.curesma.org/pfdd-meeting-summary/.

The NHC encourages FDA to recognize these community-facing impacts as meaningful, even when they do not translate immediately into a specific regulatory decision. Patient-community mobilization, shared language, better identification of unmet need, and stronger research prioritization are important outputs in their own right. These outcomes can also create the conditions for more focused and higher-quality engagement later in the development process. FDA's synthesis of docket comments should therefore treat these community-level effects as part of the overall value of PFDD, even when they are not readily captured in a regulatory timeline.

At the same time, the NHC cautions FDA against treating PFDD meetings as the only pathway for meaningful patient input. Some conditions or communities may be better served initially by smaller listening sessions, interviews, survey work, registry development, advisory groups, or other fit-for-purpose methods. The NHC encourages FDA to frame PFDD meetings as one valuable engagement mechanism within a broader continuum rather than as a de facto expectation for all patient communities.

The practical value of a PFDD meeting will often depend on whether its outputs are connected to a structured plan for use. A meeting can identify themes, generate hypotheses, surface patient-centered concepts of interest, and highlight gaps in understanding. Additional work may then be needed to assess subgroup differences, quantify preferences, validate measures, or translate patient input into trial design, endpoint selection, or regulatory strategy. FDA can help stakeholders by clarifying these pathways and by providing examples of how meeting outputs can appropriately inform follow-on work.

Using PFDD Meeting Outputs to Identify Research Gaps and Patient-Centered Evidence Needs

The NHC has previously emphasized that not all patient-reported outcomes are patient-centered, and not all patient-centered outcomes are necessarily patient-reported. A measure is patient-centered when it captures a concept that patients identify as important. PFDD meetings can help identify those concepts and can provide a starting point for determining whether existing measures are adequate, should be modified, or need to be supplemented by new measures. That distinction remains important because the use of a patient-reported measure does not, by itself, demonstrate that it reflects patients' needs and priorities.[26]

PFDD meetings can identify gaps in the scientific and clinical understanding of a disease or condition, especially related to aspects of the patient and caregiver experience, that may be underrecognized in the published literature or insufficiently captured by existing clinical outcome assessments. These gaps may involve symptoms and functional effects, such as fatigue, pain, cognitive effects, and limitations on social participation; treatment-related burdens, such as route of administration, dosing frequency, monitoring requirements, and treatment setting; and broader effects on daily life, including disruption to work or school, stigma, caregiver burden, and uncertainty

[26] National Health Council, "NHC Comments on Patient-Focused Drug Development Guidance."

about disease progression. These findings can help redirect research attention toward issues that are clinically relevant, meaningful to patients, and potentially overlooked by traditional development programs. FDA's synthesis should therefore treat these gaps as potential starting points for follow-on research and methodological refinement rather than as incidental observations.[27,28,29,30]

The NHC recommends that FDA use the information collected through this docket to develop a clearer description of how PFDD meeting findings can lead to different types of research action. For example, one meeting may identify concepts of interest that require qualitative follow-up, while another may reveal the need for patient preference research to understand tradeoffs among benefits, risks, uncertainty, convenience, and treatment burden. Another may demonstrate that natural history data or registry infrastructure is needed before a patient-centered endpoint can be developed or interpreted reliably. Public examples show PFDD-related work contributing to survey research, patient-experience data resources, rare disease evidence infrastructure, and cross-stakeholder efforts to apply PFDD concepts beyond the meeting itself. In that sense, PFDD meetings can help define the next research question as much as they answer an existing one.[31,32,33]

Additional FDA examples or explanatory materials could help stakeholders understand that the appropriate follow-on action from a PFDD meeting will depend on the intended use of the information. Evidence used to inform early community research priorities may require a different level of documentation than evidence intended to support an endpoint selection, regulatory submission, or benefit-risk assessment. The NHC encourages FDA

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[27] Cancer Support Community, *Cancer Cachexia Voice of the Patient Report* (Washington, DC: Cancer Support Community, 2022), https://www.cancersupportcommunity.org/sites/default/files/file/2022-10/CCommSupport_Cachexia_Report_Final.pdf.

[28] Lupus Foundation of America, "Lupus Community Shares Views with FDA at Historic Patient-Focused Drug Development Meeting," September 26, 2017, https://www.lupus.org/news/lupus-community-shares-views-with-fda-at-historic-patientfocused-drug-development-meeting.

[29] National Psoriasis Foundation, "The Power of the Patient Voice," accessed June 16, 2026, https://www.psoriasis.org/advance/the-power-of-the-patient-voice/.

[30] Schizophrenia & Psychosis Action Alliance, "Patient-Focused Drug Development," accessed June 16, 2026, https://sczaction.org/insight-initiative/pfdd/.

[31] EveryLife Foundation for Rare Diseases, *Guide to Patient Involvement in Rare Disease Therapy Development* (Washington, DC: EveryLife Foundation for Rare Diseases, 2022), https://everylifefoundation.org/wp-content/uploads/2022/01/Guide-to-Patient-Involvement-FINAL-COMPLETE-GUIDE-Rev.pdf.

[32] National Organization for Rare Disorders, "NORD Comments on FDA Patient-Focused Drug Development Guidance 2," January 28, 2020, https://rarediseases.org/wp-content/uploads/2020/01/NORD-2019-Comments-on-FDA-PFDD-Guidance-2.pdf.

[33] ALS Association, "ALS Focus Results: Treatment," accessed June 16, 2026, https://www.als.org/research/participate-research/als-focus-survey-program/survey-results/als-focus-results-treatment.

to preserve methodological flexibility while giving stakeholders clearer expectations regarding transparency, documentation, and evidentiary fitness for different uses. This distinction can help prevent both overinterpretation of meeting outputs and underuse of patient input that is relevant but not sufficient for a particular regulatory purpose.

The NHC also recommends that FDA treat gaps identified through PFDD meetings as actionable signals. If multiple PFDD meetings across disease areas identify recurring concerns regarding treatment burden, caregiver burden, assessment burden, trial feasibility, or underrepresentation, those cross-cutting themes may warrant additional FDA attention. Such themes should not replace disease-specific nuance, but they can help FDA and stakeholders identify areas where broader guidance, case examples, or methodological development would be useful. FDA can use this docket to identify whether such cross-cutting issues point to broader methodological needs that would benefit multiple disease communities.

Applying Patient Input to Medical Product Development and Regulatory Decision-Making

Patient input from PFDD meetings can inform medical product development by identifying unmet need, shaping research priorities, assessing the meaningfulness of potential endpoints, improving clinical trial design, uncovering barriers to recruitment and retention, and supporting more patient-centered approaches to benefit-risk evaluation. These uses are consistent with the NHC's long-standing view that patient experience data can be relevant across the medical product lifecycle rather than only at a single point in clinical development. FDA's impact framework should therefore be broad enough to capture development changes that are meaningful to patients even when they are not visible as discrete regulatory milestones.

The NHC encourages FDA to recognize both direct and indirect product-development impacts. Some PFDD meeting outputs may be used directly in discussions of endpoints, clinical outcome assessments, benefit-risk considerations, or development strategy. Other impacts may be less direct but still important, such as improving the feasibility of a trial, refining eligibility criteria, reducing participant burden, adjusting assessment schedules, improving informed consent materials, or strengthening patient-facing questionnaires and recruitment materials. FDA's assessment of impact should therefore account for operational changes that make a trial or development strategy more feasible and relevant from the patient perspective.

Patient input can be especially useful in determining whether a proposed trial design is workable in real life. For example, a protocol may appear scientifically sound but impose visit schedules, travel requirements, procedures, washout periods, monitoring obligations, or data-collection burdens that are difficult for patients and caregivers to manage. If those burdens are not identified early, they can lead to reduced enrollment and retention, missing data, and weakened interpretability of the resulting evidence. PFDD meetings and related engagement can help sponsors and researchers identify these issues before they undermine a development program.

PFDD meeting outputs may also help sponsors and researchers understand the relative importance of different treatment attributes. Patients may place substantial value on outcomes or attributes that are not always prioritized in traditional development planning, including reduced treatment burden, ability to maintain work or school activities, decreased caregiver dependence, more manageable monitoring requirements, fewer disruptions to daily life, or greater predictability of symptoms. Patient preference studies and other follow-on methods may be needed to quantify these tradeoffs, but PFDD meetings can help identify which tradeoffs should be studied. Follow-on methods can then determine how they should be weighed or measured in a particular development context.

Public examples show PFDD-related work being used to identify treatment priorities, support patient-centered clinical trial principles, inform drug and device development, and generate patient-experience data relevant to advocacy organizations, industry, regulators, and other decision makers. FDA review documents also provide context-specific examples in which patient and caregiver perspectives from an EL-PFDD meeting were considered in benefit-risk assessment, demonstrating that PFDD input may be relevant not only to upstream development planning but also to regulatory evaluation in appropriate circumstances. These examples underscore why FDA should consider patient input as part of a broader evidentiary ecosystem rather than as a single meeting report or isolated submission.[34,35,36,37,38,39]

At the same time, the NHC cautions FDA against framing regulatory impact too narrowly. A PFDD meeting should not only be considered valuable if it can be tied to a specific labeling claim or approval decision, nor should the absence of such a connection diminish the perceived value of well-designed patient engagement. If a meeting helps a sponsor select a more meaningful endpoint, reduce trial burden, design

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[34] Food and Drug Administration, *Integrated Review: NDA 215935* (Rockville, MD: Food and Drug Administration, 2022), https://www.accessdata.fda.gov/drugsatfda_docs/nda/2022/215935Orig1s000IntegratedR.pdf.

[35] Food and Drug Administration, *Integrated Review: BLA 761434* (Rockville, MD: Food and Drug Administration, 2025), https://www.accessdata.fda.gov/drugsatfda_docs/nda/2025/761434Orig1s000IntegratedR.pdf.

[36] ALS Association, "About ALS Focus," accessed June 16, 2026, https://www.als.org/research/als-focus/about-als-focus.

[37] ALS Association, "Principles for Urgent, Patient-Centered ALS Clinical Trials," accessed June 16, 2026, https://www.als.org/blog/principles-urgent-patient-centered-als-clinical-trials-latest-ongoing-effort-improve-trial.

[38] Asthma and Allergy Foundation of America, "Patient-Focused Drug Development for Asthma," accessed June 16, 2026, https://aafa.org/asthma-allergy-research/our-research/patient-focused-drug-development-asthma/.

[39] LUNGevity Foundation, "Comments on Patient-Focused Drug Development: Collecting Comprehensive and Representative Input," September 11, 2018, https://www.lungevity.org/sites/default/files/public-policy/091118-LF-Comment-Letter-PFDD-Guidance.pdf.

a more inclusive study, understand treatment tradeoffs, or improve patient-facing materials, that can represent meaningful impact even if the connection is not visible in the final regulatory record. A narrow approach would risk undervaluing the practical changes that can make a development program more patient-centered, feasible, and relevant to affected communities.

The NHC therefore recommends that FDA use this docket to identify and disseminate case examples that reflect the full range of product-development uses. Examples should include early research planning, endpoint development, clinical trial design, patient preference research, patient-facing materials, benefit-risk considerations, post-market evidence planning, and communication of treatment information. FDA could also identify examples where meeting outputs were useful but insufficient on their own, because those examples can help stakeholders understand when additional fit-for-purpose evidence is needed. Examples that show both successful uses and appropriate limitations would be particularly helpful because they would help stakeholders calibrate expectations.

Translating PFDD Insights into Clinical Practice and Patient-Clinician Communication

Although PFDD meetings are often discussed in the context of medical product development and regulatory decision-making, their impact can extend into clinical practice and patient-clinician communication. Meeting reports and related materials can help clinicians better understand aspects of disease, such as symptoms, daily-life impacts, treatment burdens, and tradeoffs that patients and caregivers identify as important. These resources can also highlight symptoms or burdens that may not surface consistently or be visible during a brief clinical encounter.

This impact may be especially important when a disease or condition has heterogeneous symptoms, episodic manifestations, invisible symptoms, stigma, caregiver dependence, or effects on daily functioning that are difficult to capture in standard clinical measures. PFDD meeting outputs can help translate lived experience into terms that are understandable to clinicians, researchers, sponsors, and regulators. They can also provide patient organizations with materials that support education, advocacy, and shared understanding within the community. Those materials may also help bridge communication gaps between what patients experience and what clinicians, researchers, or developers routinely measure.

PFDD meeting outputs may also improve shared decision-making. By clarifying which outcomes and treatment attributes matter most to patients, PFDD work can help clinicians and patients discuss benefits, risks, side effects, uncertainty, route of administration, monitoring requirements, and treatment burden in a more patient-centered way. In this sense, PFDD meetings may contribute to clinical practice even when they are not designed as clinical guideline exercises. When used in this way, PFDD outputs can support individualized discussions about which treatment characteristics matter most to a patient or caregiver.

Public examples show PFDD meetings and related reports being framed as resources for clinicians, researchers, health care leaders, patient communities, FDA, and product developers. These materials can help translate patient experience into education, care priorities, and shared understanding across stakeholders. The NHC encourages FDA to recognize these impacts as meaningful, even if they are difficult to quantify or attribute to a single downstream decision and to consider these educational and communication uses as part of the broader impact of PFDD meetings, even where the effect is indirect.[40,41,42,43]

The NHC recommends that FDA consider whether future PFDD meeting reports or impact summaries could include a section designed to support clinical translation. Such a section could identify patient-described symptoms, impacts, treatment burdens, and communication needs that may be relevant to clinical care, while making clear that the report should be interpreted in light of its scope, methods, participant characteristics, and limitations. Such an approach would not convert PFDD reports into practice guidelines, but it could make their clinical relevance easier for stakeholders to understand. A clinical-translation section could help ensure that meeting findings are applicable for clinicians and patient communities without overstating the evidentiary role of the report.

FDA may wish to consider asking patient organizations, clinical societies, and academic researchers to provide examples of how PFDD meeting outputs have informed clinician education, patient education, shared decision-making tools, care priorities, continuing education, or disease-awareness initiatives. These examples would help FDA and stakeholders better understand the role of PFDD meetings outside formal regulatory submissions and better document how patient experience information moves from regulatory engagement into care delivery, education, and communication. Because these clinical and educational applications may be less visible to FDA than formal submissions, this docket is an appropriate opportunity to assess their scope and significance.

Building Patient-Community Capacity and Durable PFDD Resources

PFDD meetings can produce broader effects that are important to the long-term success of patient-centered medical product development. Organizing a PFDD meeting often requires patient organizations to build or strengthen capabilities in outreach, facilitation, survey design, data collection, report development, dissemination,

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[40] Cancer Support Community, “Cancer Cachexia PFDD,” accessed June 16, 2026, https://www.cancersupportcommunity.org/cachexia-pfdd.

[41] Lupus Foundation of America, “Patient-Focused Drug Development Meeting.”

[42] Arthritis Foundation, *Juvenile Idiopathic Arthritis Voice of the Patient*.

[43] Asthma and Allergy Foundation of America, “Little Airways, Big Voices Press Release,” September 15, 2021, https://aafa.org/wp-content/uploads/2022/08/aafa-press-release-little-airways-big-voices-childhood-asthma-15-september-2021.pdf.

stakeholder coordination, and follow-up engagement. Those capabilities can continue to benefit patient communities after the meeting has concluded by positioning patient organizations to participate more effectively in future research and policy discussions.

PFDD meetings can also create durable public resources. Voice of the Patient reports, meeting summaries, survey reports, and related materials can help researchers, sponsors, clinicians, regulators, and patient organizations understand the patient experience without requiring every stakeholder to begin from scratch. These resources may be especially important in rare diseases and other areas where evidence is limited, patient communities are dispersed, or traditional clinical literature does not fully reflect the lived experience of patients and caregivers. These resources can also help lower-resource stakeholders avoid duplicating work while identifying where additional engagement is still needed.[44,45,46]

The NHC encourages FDA to view these durable resources as part of the public infrastructure for patient-centered evidence. A searchable repository or compendium of PFDD meeting outputs, related reports, and impact summaries could help stakeholders identify existing work, understand what has already been learned, avoid duplication, and determine where additional research is needed. Such a resource could also support lower-resource patient organizations by making examples and templates more accessible. The practical value of such a resource would depend on whether it is easy to navigate and organized in a way that supports both disease-specific and cross-cutting learning.

At the same time, the NHC urges FDA to ensure that any effort to standardize impact reporting does not unintentionally create burdens that discourage participation. Patient organizations vary significantly in staffing, technical capacity, disease prevalence, fundraising base, and research infrastructure. A reporting framework that is too complex could be difficult for smaller or lower-resource organizations to use. The NHC therefore recommends an optional, modular approach that supports consistency without imposing unrealistic expectations.

Such a template could include sections on meeting objectives, populations reached, recruitment approaches, key themes, identified gaps, follow-on activities, known limitations, and examples of downstream use. It should also allow organizations to indicate when information is not available or when impact is still emerging. A flexible template would help FDA and stakeholders compare examples while respecting the diversity of patient communities and engagement models. This approach would allow

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[44] National Organization for Rare Disorders, “Patient-Focused Drug Development,” accessed June 16, 2026, https://rarediseases.org/living-with-a-rare-disease/patient-focused-drug-development/.

[45] EveryLife Foundation for Rare Diseases, “Rare Disease PFDD Compendium,” accessed June 16, 2026, https://everylifefoundation.org/pfdd-compendium/.

[46] EveryLife Foundation for Rare Diseases, *Guide to Patient Involvement in Rare Disease Therapy Development*.

FDA to learn more consistently from PFDD work while respecting the reality that not every impact can be fully measured at the same point in time.

Representativeness, Transparency, and Limits of Interpretation

The NHC encourages FDA to remain attentive to representativeness and transparency when evaluating PFDD meeting impacts. PFDD meetings are often designed to elicit rich patient and caregiver perspectives, not to produce statistically representative estimates for every affected population. That does not diminish their value, but it does mean that outputs should be interpreted in light of the meeting objectives, methods, recruitment strategy, participant characteristics, and limitations. The goal should be to make PFDD outputs more interpretable and useful, not to impose a single rigid standard that would be inappropriate across all disease areas and engagement models.

The NHC's prior work emphasizes that the appropriate representativeness standard depends on the purpose of the engagement and the question being addressed. For some purposes, a small number of in-depth narratives may provide sufficient insight. For other purposes, broader survey work, targeted outreach, stratified sampling, or additional follow-up may be needed. The NHC encourages FDA to promote transparency about these distinctions rather than applying a single rigid expectation across all PFDD activities.[47,48,49]

The NHC also encourages FDA to support documentation of populations that may not have been adequately reached. For example, PFDD meetings may underrepresent people with limited English proficiency, low health literacy, limited internet access, limited mobility, high caregiving responsibilities, advanced disease, rural residence, lower income, or mistrust of research or regulatory processes. In some disease areas, pediatric patients, caregivers, older adults, people with cognitive limitations, or people with substantial disability may require tailored approaches. Documenting these gaps can help stakeholders plan targeted follow-up engagement rather than assuming that a single meeting captured all relevant perspectives.

The NHC recommends that FDA promote transparency regarding these limitations while preserving the value of PFDD meeting outputs. A meeting report that clearly describes its scope and limitations is more useful than one that implies broader representativeness than the methods support. Similarly, an impact framework should distinguish among meeting outputs that identify themes, generate hypotheses, support follow-on research, or contribute to regulatory evidence package. This approach would

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[47] National Health Council, *Tackling Representativeness*.

[48] National Health Council, *Patient Perspectives on Disease Impact and Treatment Options: A Stratification Tool* (Washington, DC: National Health Council, 2016), https://nationalhealthcouncil.org/wp-content/uploads/2019/12/NHCPatientInformationToolandinstructions_0.pdf.

[49] National Health Council, *Implementation Manual: How to Operationalize the National Health Council's Patient Information Tool* (Washington, DC: National Health Council, 2016), https://nationalhealthcouncil.org/wp-content/uploads/2019/12/Patient-Information-Tool-Manual.pdf.

make PFDD outputs more credible and easier to use, while preserving the central role of patient and caregiver narratives.

This approach would also help protect against both overuse and underuse of PFDD meeting outputs. Overuse can occur if stakeholders treat a meeting report as sufficient for purposes that require additional evidence. Underuse can occur if stakeholders discount patient narratives because they are not statistically representative in a traditional sense, even when the intended use is concept elicitation, hypothesis generation, or identification of meaningful impacts. FDA can help stakeholders navigate this balance by providing examples that connect methods, limitations, and intended use.

Capturing Regulatory and Non-Regulatory Impact

The NHC supports FDA's interest in understanding PFDD impacts that occur outside specific regulatory decisions. A narrow focus only on approval decisions, labeling, or formal submissions would miss many of the ways PFDD meetings have influenced patient communities, research priorities, sponsor planning, clinical trial design, and stakeholder engagement. At the same time, the NHC encourages FDA to capture regulatory impacts where they are visible and to clarify how such examples should be understood. For that reason, FDA's assessment of impact should be broad enough to capture how PFDD meetings influence decisions, relationships, and evidence-generation activities over time.

In some cases, patient and caregiver perspectives from PFDD meetings have been reflected in FDA review materials or benefit-risk discussions. These examples are important because they demonstrate that PFDD outputs can have regulatory relevance. However, the NHC encourages FDA to avoid implying that a meeting has impact only when it is cited in a review document. The patient-centered value of PFDD is broader than formal citation.[50,51]

The NHC recommends that FDA describe several categories of impact. These could include community impact, such as mobilization, shared language, and prioritization; research impact, such as follow-on studies, registries, patient preference work, or clinical outcome assessment development; development impact, such as protocol design, endpoint selection, recruitment, retention, or patient-facing materials; regulatory impact, such as benefit-risk assessment or review discussions; and clinical or educational impact, such as clinician education, patient education, or shared decision-making resources. A structured taxonomy of impact would also make it easier for stakeholders to submit comparable examples without forcing very different activities into a single category. That structure would also help FDA identify where future examples, templates, or educational materials may be most useful.

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[50] Food and Drug Administration, *Integrated Review: NDA 215935*.

[51] Food and Drug Administration, *Integrated Review: BLA 761434*.

Categorizing impacts in this way would help stakeholders understand that PFDD meetings can produce value through multiple pathways. It would also help FDA identify where evidence of impact is currently difficult to capture. For example, a sponsor may use meeting findings to change a protocol, but that change may not be attributed publicly to the meeting. A patient organization may use meeting findings to build a registry or survey program, but FDA may not see that work unless it is submitted or described in a docket. A clinician education effort may rely on a Voice of the Patient report, but that use may not be connected back to FDA.

The NHC encourages FDA to consider whether an ongoing voluntary mechanism is needed to capture these impacts over time. A public docket is useful, but many impacts emerge years after a meeting. An optional annual or periodic update process, a structured impact template, or an FDA-maintained compendium could make it easier to capture evolving uses without requiring a new RFI each time FDA seeks information, provided that any such mechanism remains voluntary, minimally burdensome, and limited to information stakeholders are able and willing to share. FDA could design such a mechanism so that participation remains voluntary and flexible, while still making it easier to learn from impacts that emerge over time.

Ensuring PFDD is Accessible to Patient Organizations with Varying Capacity

The NHC urges FDA to remain attentive to differences in patient organization capacity. PFDD meetings can be valuable, but organizing an EL-PFDD meeting, preparing a Voice of the Patient report, conducting supplemental survey research, and tracking downstream impact can require significant resources. Smaller organizations, newer disease communities, and communities with limited funding may not be able to undertake the same scope of activity as larger organizations. This capacity issue should be considered part of the patient-centeredness of any future framework because unrealistic expectations can exclude the very communities that most need better evidence.

The NHC cautions FDA against creating a practical expectation that only communities with the resources to conduct large meetings or extensive follow-on research can provide meaningful patient input and suggesting that every development program must generate the same type or volume of PFDD-related documentation. A patient-centered system should allow for multiple pathways to communicate patient experience, including lower-resource methods that are still structured, transparent, and fit for purpose. These may include smaller listening sessions, targeted interviews, community surveys, collaboration with academic researchers, use of registries, or participation in cross-disease initiatives. FDA’s communications should reinforce that the credibility of patient input depends on fit-for-purpose methods and transparent documentation, not simply on the size or cost of the engagement activity.

The NHC recommends that FDA develop practical resources tailored to lower-resource organizations. These could include sample meeting objectives, suggested documentation elements, simplified report templates, examples of lower-resource engagement methods, and guidance on how to describe limitations. FDA could also identify ways for patient organizations to build on existing PFDD reports, public

datasets, or cross-disease resources without duplicating prior work. These tools would be especially valuable if they helped organizations describe what they did, what they learned, what limitations remain, and what follow-on work may be needed.

This issue is particularly important because the communities most in need of better patient-centered evidence may not always have the strongest infrastructure to generate it. Rare disease communities, communities affected by stigma, communities with substantial health disparities, and communities with high caregiver burden may need more flexible and supportive pathways for engagement. The NHC encourages FDA to structure the PFDD impact framework to strengthen inclusion rather than reinforcing differences in organizational capacity. A flexible approach would help ensure that FDA's PFDD infrastructure supports equity, rather than unintentionally privileging communities with greater organizational capacity.

Recommendations to Strengthen the Use and Visibility of PFDD Meeting Impacts

As FDA reviews the information submitted to this docket, the NHC encourages the Agency to focus on how PFDD meeting outputs can be made more useful in practice. The central question should not be only whether PFDD meetings have had impact, but how FDA and stakeholders can better document, communicate, and build on that impact. FDA may wish to consider how future resources can reduce uncertainty for stakeholders who want to use PFDD outputs appropriately but are unsure what level of follow-on evidence is needed. The recommendations below are intended to support practical orientation while preserving the methodological flexibility that is essential to patient-centered evidence generation. The NHC recommends that FDA:

1. Develop a public PFDD impact framework by synthesizing docket comments into a description of the common pathways through which PFDD meetings affect patient-community engagement, research priorities, medical product development, regulatory decision-making, clinical practice, and patient-community capacity. The framework should recognize both direct and indirect impacts and should avoid treating formal regulatory citation as the only meaningful measure of value. It should also help stakeholders distinguish between meeting outputs, follow-on evidence generation, and downstream uses of patient input. A public framework would be most useful if it included examples, common terminology, and practical considerations for interpreting impact in light of each meeting's purpose and limitations.
2. Publish practical case examples, including de-identified, hypothetical, or real-world examples showing how PFDD meeting input has been used across the medical product lifecycle. Examples should include endpoint development, clinical trial design, patient preference research, benefit-risk assessment, recruitment and retention planning, patient-facing materials, post-market evidence generation, patient education, clinician education, and shared decision-making. The examples should describe not only the final use of patient input, but also the intermediate steps that connect meeting findings to a development, research, clinical, or communication activity. This level of detail would help stakeholders understand what a credible pathway from meeting output to downstream use can look like in practice.

3. Clarify the relationship between PFDD meetings and follow-on evidence generation by reiterating that PFDD meetings are valuable but are one method among many. Meeting outputs may need to be supplemented by additional qualitative research, quantitative research, patient preference studies, natural history studies, registries, or other methods depending on the intended use. Such clarification would reinforce that this need for follow-on evidence does not diminish the value of the meeting itself. Rather, it reflects the importance of matching the method, level of documentation, and evidentiary expectations to the decision or activity the information is intended to inform.
4. Consider creating an optional impact reporting template that patient organizations and other stakeholders can use to document meeting outputs and downstream impacts in a standardized but flexible manner, without creating new regulatory expectations, mandatory reporting obligations, or pressure to disclose confidential information. The template should be modular and should not create unnecessary burden for lower-resource organizations. It should allow organizations to describe impacts that are still emerging, difficult to quantify, or not tied to a specific regulatory decision. Such a template could improve consistency while preserving the flexibility needed for different patient communities, disease areas, and engagement models.
5. Promote transparency regarding methods and limitations by encouraging PFDD meeting organizers to describe objectives, recruitment strategies, participant characteristics, data collection methods, known limitations, and plans for follow-up engagement. This information will help stakeholders interpret meeting outputs appropriately and determine where additional evidence may be needed. Transparency should not be treated as a mechanism for discounting patient input, but as a way to make that input more usable and appropriately contextualized. FDA could also encourage stakeholders to describe populations that were not reached and the steps that may be needed to understand those perspectives more fully.
6. Support lower-resource engagement pathways by providing examples of structured, practical approaches to collecting and communicating patient input when a large EL-PFDD meeting or extensive follow-on research program is not feasible. Not every community can conduct a large EL-PFDD meeting, but many can contribute meaningful patient experience information through fit-for-purpose methods. FDA could make clear that smaller or lower-resource approaches can still be valuable when they are well documented, appropriately scoped, and transparent about limitations. This is especially important for communities with limited infrastructure, small patient populations, or substantial access barriers.
7. Improve access to PFDD resources by exploring a searchable repository or compendium of publicly available and voluntarily submitted PFDD meeting reports, Voice of the Patient reports, impact summaries, templates, and examples. Such a resource would help stakeholders identify existing work, learn from prior examples, and avoid unnecessary duplication. It could also help patient organizations and researchers understand where follow-on work may be most useful. To be effective, the repository should be easy to navigate, regularly maintained, and organized in a way that supports both disease-specific searches and cross-cutting learning.

8. Continue stakeholder engagement by working with patients, caregivers, patient organizations, researchers, sponsors, clinicians, and review staff as it determines how to use the information collected through this docket. The value of the effort will depend on whether future resources are practical, accessible, and responsive to the needs of those expected to use them. Continued engagement will also help FDA identify where its approach may need refinement as PFDD methods, data sources, and stakeholder needs evolve. The NHC encourages FDA to treat this RFI as one step in an ongoing process of making patient input more visible, interpretable, and useful across the medical product lifecycle.

Using the Docket to Build a More Usable PFDD Infrastructure

The NHC encourages FDA to view this docket as part of a broader infrastructure-building effort. PFDD meetings have helped normalize the idea that patient input is relevant to medical product development and regulatory decision-making. The next stage should focus on making the outputs of those meetings easier to locate, interpret, connect to follow-on evidence, and use appropriately. Without that additional infrastructure, the practical value of PFDD meeting outputs may remain uneven across disease areas, sponsors, review divisions, and patient communities.

A more usable PFDD infrastructure would help address several recurring challenges. First, stakeholders may not know whether relevant PFDD meeting reports or Voice of the Patient reports already exist for a disease area or related condition. Second, when those resources do exist, stakeholders may not know how to assess their scope, methods, limitations, and appropriate uses. Third, patient organizations may not have a consistent way to describe downstream impact or to update FDA and other stakeholders as additional work emerges. Fourth, sponsors and researchers may vary in how they incorporate PFDD outputs into development planning, even where the information is directly relevant.

The NHC recommends that FDA use the comments submitted to this docket to identify the common elements that make PFDD outputs more applicable. Those elements could include a clear statement of meeting objectives, description of recruitment and outreach methods, summary of participant characteristics, discussion of limitations, identification of key patient-described symptoms and impacts, description of treatment burdens and unmet needs, and suggested areas for follow-on work. While these elements should not be treated as rigid requirements, they would make PFDD materials easier to evaluate and apply and help stakeholders understand what information is available and how it should be interpreted.

FDA may wish to consider whether future PFDD impact summaries can distinguish between immediate outputs and longer-term effects. Immediate outputs may include meeting transcripts, polling results, written comments, survey summaries, and Voice of the Patient reports. Longer-term effects may include new research collaborations, patient preference studies, registry development, changes to trial design, development or modification of clinical outcome assessments, patient education materials, clinician education, or regulatory use. Distinguishing these categories would help FDA and

stakeholders understand that PFDD impact often develops over time rather than ending with publication of a meeting report.

The NHC also encourages FDA to consider how PFDD outputs can be made more accessible to non-specialist users. Many patients, caregivers, and community stakeholders may not be familiar with regulatory terminology, clinical outcome assessment methodology, or benefit-risk frameworks. If PFDD reports and impact summaries are written only for technical audiences, their value to patient communities may be limited. The NHC encourages FDA to support accessible summaries that explain what was learned, how the information may be used, what additional work may be needed, and how patients and caregivers can remain engaged.

This recommendation is consistent with the NHC's broader view that patient-centered processes should be usable in practice. A PFDD report that exists but is difficult to find, interpret, or connect to next steps may have limited impact, while a report that is accessible, transparent, and linked to follow-on actions can serve as a durable resource for the patient community and for the broader development ecosystem. The NHC encourages FDA to assess whether PFDD reports and related outputs can support follow-on work, not only whether they were produced.

Methodological Flexibility and Fit-for-Purpose Use

The NHC recommends that FDA continue to emphasize fit-for-purpose use of patient experience information. PFDD meetings can provide important qualitative insight, but the appropriate evidentiary role of a meeting output depends on the question being asked. A meeting report may be well suited to identifying concepts of interest, describing lived experience, identifying areas of unmet need, or highlighting potential treatment burdens. It may not, by itself, be sufficient to quantify preferences, validate a measure, estimate prevalence of a symptom, or support all aspects of a regulatory submission. FDA can help stakeholders by describing these distinctions clearly.

This type of clarity would be particularly useful for patient organizations and sponsors seeking to determine what work should follow a PFDD meeting. For example, if a meeting identifies fatigue, pain, cognitive impairment, mobility, or caregiver burden as key concerns, additional qualitative work may be needed to characterize those concepts. A patient preference study may be appropriate if stakeholders need to understand tradeoffs among treatment attributes. Poorly understood disease progression may warrant natural history research or registry. If an existing outcome measure appears incomplete or insufficiently patient-centered, additional work may be needed to determine whether it can be adapted or whether a new measure is warranted.

The NHC encourages FDA to provide examples of how these decisions can be made without becoming overly prescriptive, recognizing that disease areas differ significantly in prevalence, severity, heterogeneity, available treatments, existing evidence, patient organization capacity, and development landscape: A rare disease community with limited data and a small number of identifiable patients may need a different approach than a common chronic disease with multiple therapies and extensive published

literature. A pediatric condition may require different engagement methods than an adult condition. A condition with substantial caregiver involvement may require a different evidentiary plan than one in which patients can report directly for themselves.

The NHC recommends that FDA recognize the value of lower-resource approaches if they are transparent and appropriately matched to the intended use. The NHC would be concerned if the field moved toward a practical expectation that only large, highly resourced patient organizations can generate patient experience information that is taken seriously. A fit-for-purpose framework should allow for smaller, staged, and collaborative approaches while making clear when additional evidence is needed for higher-stakes uses. This flexibility would help ensure that meaningful patient input is not limited to the communities with the greatest organizational infrastructure.

The NHC further cautions the FDA against creating an implied hierarchy in which PFDD meetings are treated as inherently superior or inferior to other methods. A meeting may be the right tool for one purpose and not another. Surveys, interviews, focus groups, patient preference studies, registries, natural history studies, digital data sources, advisory groups, and patient-led research may all be appropriate in different circumstances. The central question should be whether the approach is transparent, ethical, patient-centered, and fit for the decision or activity it is intended to inform.

Consistency Across FDA and Across Stakeholders

The NHC encourages FDA to consider how the information collected through this docket can support greater consistency across FDA review contexts and stakeholder practice. Patients and patient organizations invest substantial effort in PFDD meetings and related activities. That work is more likely to produce meaningful impact when stakeholders have a shared understanding of how PFDD outputs can be used, what additional evidence may be needed, and how patient experience information should be documented. A shared understanding would make it more likely that patient experience information is used consistently rather than episodically.

Consistent use does not require a rigid or one-size-fits-all approach. Rather, it depends on clearer expectations regarding the questions stakeholders should ask when using PFDD outputs. Those questions may include: What was the objective of the meeting? Who participated and who may not have been reached? What symptoms, impacts, and treatment burdens were identified? Were findings supplemented by surveys or other data collection? What follow-on research occurred? How were findings considered by sponsors, researchers, clinicians, or FDA? What limitations should be considered before using the information for a specific purpose?

A common set of questions would allow sponsors and researchers to use PFDD outputs more responsibly and help patient organizations communicate their work more effectively. It could also support greater consistency in FDA interactions with patient organizations and sponsors. If different review divisions or programs vary in how they encourage, interpret, or request patient experience information, stakeholders may face uncertainty about what level of evidence is useful. FDA can reduce that uncertainty by

sharing practical examples and by clarifying how PFDD meeting outputs can fit into broader development and review discussions.

The NHC also recommends that FDA consider how PFDD meeting outputs are communicated internally. Patient experience information may be most useful when review teams can understand not only that a meeting occurred, but what it showed, how the information was collected, and how it relates to the development question at hand. FDA may wish to consider whether additional internal tools or summary formats could help review staff access and interpret PFDD outputs more consistently. Such internal communication tools could also help reduce variation in how PFDD outputs are considered across review contexts.

More broadly, the NHC urges FDA to treat PFDD impact as part of the Agency's continued work to make patient experience data a meaningful component of medical product development and regulatory decision-making. The goal should not be a separate PFDD process that exists alongside development, but a more integrated approach in which patient input informs the questions being asked, the outcomes being measured, and the decisions being made. That integrated approach is consistent with NHC's view that patient engagement should inform the substance of development, not simply satisfy a process expectation. The NHC also encourages FDA to use this docket to identify where additional guidance or examples would make patient input easier to use.

Avoiding Unintended Burden from Impact Reporting

The NHC supports FDA's effort to collect information on PFDD meeting impact. At the same time, the Agency should ensure that any future impact-reporting expectations do not create unnecessary burden for patient organizations. Patient organizations often operate with limited staff and resources while supporting broad community needs, including education, advocacy, research engagement, peer support, and policy work. Additional documentation expectations should be designed to help organizations communicate impact, not to create a new administrative barrier to engagement.

The NHC recommends that FDA pursue a flexible and voluntary approach to reporting meeting impact, such as a template or reporting framework that allows patient organizations to provide information at different levels of detail depending on available resources and the maturity of downstream work. For example, an organization may be able to describe immediate outputs and planned follow-up activities shortly after a meeting, but more substantial information on development impact or clinical education may not be available until years later. For that reason, a reporting framework should accommodate that timing rather than assuming that every impact will be known at the same point in the process.

The NHC urges FDA to avoid requiring patient organizations or sponsors to disclose information that may be confidential, proprietary, or dependent on relationships with sponsors, researchers, or clinical partners. Some impacts may involve early-stage research planning or development discussions that cannot be fully described publicly. The NHC encourages FDA to allow stakeholders to describe impacts at an appropriate

level of generality while still making useful information available to the public where possible. This approach would preserve transparency while recognizing that some downstream uses of patient input cannot be described in full publicly.

The NHC also recommends that FDA avoid interpreting absence of documented impact as lack of value. Some communities may not have the capacity to track downstream effects, while other impacts may occur informally or without notice to the patient organization that hosted or contributed to the meeting. Some outcomes may be indirect, such as a change in stakeholder understanding, a shift in research priorities, or improved patient-clinician communication. The NHC encourages FDA to use impact reporting to illuminate value, not to create a narrow scorecard that could disadvantage lower-resource communities.

A well-designed impact framework can help FDA and stakeholders understand how PFDD meetings are used while preserving the trust and flexibility that make patient engagement possible. The NHC urges FDA to work with patient organizations before finalizing any template, repository, or reporting approach to ensure that the resulting tools are feasible, useful, and aligned with patient community needs. Stakeholder testing would help ensure that any framework is both conceptually sound and usable in the settings where patient organizations and sponsors will apply it. The resulting tools should help stakeholders interpret PFDD outputs without creating a new administrative exercise.

Suggested Categories for FDA's PFDD Impact Framework

To make the information submitted to this docket more useful, the NHC recommends that FDA organize examples of PFDD impact into categories that reflect how patient input is used. A single meeting may have more than one type of impact, and some impacts may occur years after the meeting. A categorized framework would help stakeholders identify patterns without requiring every example to fit into a single narrative. A categorized approach would give FDA a clearer way to summarize docket input and identify where future tools are needed.

The first category should focus on patient-community impact and the ways PFDD meetings strengthen a community's ability to identify, communicate, and act on shared priorities. Examples may include community mobilization, creation of shared terminology, identification of unmet needs, outreach to underrepresented groups, development of patient and caregiver education materials, and refinement of patient organization research or advocacy priorities. These impacts matter because patient organizations often connect patients, caregivers, researchers, clinicians, sponsors, and regulators. A PFDD meeting can strengthen that connective function even before it affects a specific development program.

The second category should focus on research impact, including the ways PFDD meetings identify unanswered questions, reveal where existing evidence does not adequately capture the patient experience, and shape future evidence generation. These impacts may include generation of new research questions, identification of evidence gaps, development of surveys or qualitative research studies, creation or

expansion of registries, initiation of patient preference or natural history research, and investigation of disease heterogeneity. FDA could encourage stakeholders to describe both what the meeting found, and what research followed from those findings.

The third category should be medical product development impact. This includes endpoint selection, clinical outcome assessment development or modification, target product profile considerations, clinical trial design, eligibility criteria, visit schedules, assessment timing, recruitment and retention strategies, decentralized or hybrid trial considerations, patient-facing materials, and approaches to reducing participant burden. The NHC encourages FDA to include examples showing how patient input influenced practical development decisions, even when those changes are not clearly visible in a final label or approval document.

The fourth category should focus on regulatory impact, including the ways patient experience information is reflected in FDA interactions, benefit-risk discussions, or review materials where such examples are publicly available, voluntarily submitted, or otherwise appropriate to describe. This category would include publicly described or voluntarily submitted examples involving use of PFDD meeting outputs in FDA-sponsor interactions, benefit-risk assessment, review documents, advisory committee materials, labeling discussions, post-market evidence planning, or other regulatory contexts, where relevant and appropriate. The NHC encourages FDA to acknowledge that not every PFDD meeting will have a clearly traceable regulatory impact, and that direct citation in a review document should not be treated as the only meaningful indicator of value. Where such impact is visible, examples can help the field understand how patient experience information is considered in practice. These examples should be presented in a way that is informative without suggesting that all PFDD meetings must produce direct regulatory citations.

The fifth category should be clinical and educational impact. This includes clinician education, patient-clinician communication, shared decision-making resources, patient education, disease awareness, and clinical research training. PFDD meeting outputs can help clinicians understand patient-described disease burden and treatment tradeoffs in ways that may not be apparent from traditional clinical literature. These impacts should be recognized even where they are outside FDA’s direct regulatory role.

The sixth category should be systems and capacity impact. This includes development of patient organization infrastructure, cross-stakeholder partnerships, methodological tools, templates, repositories, and other resources that make future patient engagement easier or more effective. This category is especially important because a PFDD meeting may leave behind durable capacity that supports multiple future projects, including projects not yet identified at the time of the meeting. Recognizing systems and capacity impact would help FDA capture the long-term infrastructure benefits of PFDD work.

The NHC recommends that FDA use these categories as a flexible organizing structure rather than as a mandatory checklist. The goal should be to help stakeholders describe the range of impacts that may flow from PFDD meetings while preserving room for disease-specific and community-specific differences. Over time, this structure could also help FDA identify areas where additional guidance, examples, or stakeholder education

may be needed. A flexible structure would be especially useful where impacts are still emerging or where a meeting contributes to several types of downstream work.

Practical Considerations for Patient Organizations, Sponsors, Researchers, and Review Staff

The NHC encourages FDA to consider the different needs of the stakeholders who may use PFDD meeting outputs: Patient organizations may need tools that help them plan meetings, document findings, explain limitations, identify follow-on work, and communicate impact to their communities and partners. Sponsors may need examples that help them understand how to incorporate PFDD outputs into development planning, evidence generation, and FDA interactions. Researchers may need clarity regarding how PFDD findings can inform study design, sampling, concept elicitation, and interpretation of patient experience data. FDA review staff may need concise summaries that connect patient-described priorities to the regulatory questions before them.

Because these audiences have different needs, the NHC encourages FDA to consider a layered communication approach that allows PFDD outputs to be tailored to different users without sacrificing methodological transparency. A detailed Voice of the Patient report may be appropriate for researchers, sponsors, and regulatory staff, while a shorter plain-language summary may be more useful for patients, caregivers, and community members. A structured impact summary may help FDA and external stakeholders understand how the meeting influenced subsequent work, and a methods appendix may help those who need to assess representativeness, limitations, and intended use.

The NHC also encourages FDA to promote clarity regarding the role of patient organizations in follow-on activities. Patient organizations may be well positioned to identify unmet needs, convene communities, support recruitment, advise on patient-facing materials, define terms commonly used by patients, and help interpret whether outcomes are meaningful. However, patient organizations may not have the resources or regulatory role to carry out every follow-on study or development activity. The NHC recommends that FDA support collaboration while avoiding any implication that patient organizations alone are responsible for translating meeting outputs into regulatory-grade evidence or that sponsors must rely on a particular PFDD mechanism when other fit-for-purpose approaches may be appropriate.

Sponsors and researchers should also be encouraged to consider early engagement with patient organizations, where appropriate, so that patient input can shape decisions rather than simply validate decisions that have already been made. A PFDD meeting report may be useful late in development, but the greatest practical value will likely occur if patient input informs study design, endpoint strategy, assessment burden, and recruitment planning before key decisions are locked in. FDA can reinforce this point through case examples that show the difference between early, iterative use of patient input and later, more limited use.

For FDA review staff, the NHC recommends that the Agency consider whether summaries of PFDD meeting outputs can be presented in a format that is concise, decision-relevant, and transparent regarding limitations. Review teams may benefit from a structured summary that identifies the disease burden described by patients, treatment burdens, meaningful outcomes, tradeoffs, unmet needs, and priorities for follow-on evidence generation. Such a summary should not replace full reports, but it could help make patient experience information easier to consider during review and related regulatory discussions. This type of summary could support more consistent consideration of patient input without requiring review staff to search across multiple long reports.

The NHC also encourages FDA to consider whether additional training or internal resources may be useful to support consistent interpretation of PFDD outputs. As the number of PFDD meetings and related patient experience data resources grows, review staff may encounter information collected through varied methods, presented in multiple formats, and developed for different purposes. Internal tools that help staff assess intended use, methodological transparency, and relevance to the regulatory question could support more consistent and appropriate consideration of patient input, so long as those tools are used to support consistency and do not create new evidentiary expectations outside FDA guidance or established regulatory processes. Such tools would be most useful if they preserved flexibility while helping reviewers understand whether the information is fit for the question being considered.

Building on Cross-Disease Learning While Preserving Disease-Specific Nuance

PFDD meetings are valuable in part because they capture disease-specific patient experience. The NHC encourages FDA to preserve that disease-specific nuance as it considers whether broader lessons can be drawn across multiple PFDD meetings on different disease states. At the same time, the NHC encourages FDA to look across PFDD meetings for recurring themes that may warrant broader attention. Across many conditions, patients and caregivers describe daily-life impacts, treatment burden, caregiver responsibilities, uncertainty, mental and emotional effects, difficulty participating in work or school, and tradeoffs between potential benefit and tolerability. These themes do not eliminate the need for disease-specific evidence, but they can inform broader FDA thinking about patient-centered development.

Cross-disease learning may be especially useful in areas such as assessment burden, caregiver input, pediatric engagement, patient preference methods, decentralized trial design, accessibility of patient-facing materials, and engagement of underrepresented communities. FDA could use examples from this docket to identify where additional methodological resources would be helpful across disease areas. For example, if multiple comments describe difficulty translating meeting findings into patient-centered endpoints, FDA could consider developing additional guidance on that process. If multiple comments describe challenges with documenting impact, FDA could develop a simpler reporting template.

Cross-disease learning also benefits lower-resource organizations that can utilize examples and templates developed by other patient communities. A patient

organization preparing to collect patient experience information should not have to start from scratch if similar communities have already developed useful methods, templates, or reports. A searchable repository of PFDD outputs and impact summaries could allow organizations to learn from one another while adapting approaches to their own communities. This type of shared learning would be particularly valuable for organizations that are building patient-experience evidence capacity over time.

At the same time, the NHC encourages FDA to avoid overgeneralizing across conditions, which vary significantly in symptoms, disease progression, available treatments, caregiver roles, social context, cultural considerations, and benefit-risk tradeoffs. Cross-disease themes can help identify common methodological and implementation questions, but they should inform, not replace, engagement with the affected community in a specific disease area.

The NHC therefore recommends that FDA's impact framework include both disease-specific examples and cross-disease insights. This approach would help stakeholders understand how PFDD meetings have affected individual communities while also identifying broader opportunities to improve the PFDD ecosystem. Combining disease-specific examples with cross-cutting lessons would help FDA avoid both overgeneralization and unnecessary duplication. The NHC therefore encourages FDA to organize any repository or compendium in a way that allows stakeholders to identify both disease-specific PFDD outputs and cross-cutting methodological or implementation lessons.

Communicating Impact Without Overstating Causality

The NHC recommends that FDA communicate PFDD impacts in ways that are useful without overstating causality. In many cases, a PFDD meeting may contribute to a later activity alongside other sources of evidence, stakeholder engagement, scientific developments, or changes in the development landscape. It may not be possible or appropriate to say that the meeting alone caused a particular research initiative, endpoint decision, or regulatory discussion. That limitation should not prevent FDA from recognizing meaningful contributions.

A practical impact framework should therefore allow stakeholders to describe different levels of connection: Some examples may show direct use, such as a meeting report cited in a review document or used to inform a specific development question. Other examples may show contributory use, such as meeting findings that helped shape a survey, registry, patient preference study, or patient-facing material. Still others may show contextual use, such as a meeting that improved stakeholder understanding of disease burden or helped a community organize priorities for future work.

This approach would better reflect how patient-centered evidence is used in medical product development and regulatory decision-making. Rather than serving as a single input, patient experience information may shape research questions, inform interpretation of other data, or highlight concerns that should be evaluated through additional research. These contributions remain valuable even if they cannot be linked to a single causal claim.

The NHC recommends that FDA encourage commenters and future users of any impact template to be clear about the nature of the connection between a PFDD meeting and a downstream activity. For example, stakeholders could indicate whether a PFDD output directly informed a decision, contributed to a broader evidence-generation effort, supported community prioritization, or provided context for clinical or regulatory discussions. This type of transparency would strengthen the credibility of the impact framework and avoid unrealistic expectations regarding attribution. A clear description of attribution would help stakeholders distinguish direct effects from broader contributions to evidence generation or community strategy.

The NHC cautions FDA against using impact examples in a way that creates pressure for every PFDD meeting to produce the same types of downstream outcomes. A meeting in one disease area may lead to endpoint work; another may reveal the need for basic natural history research; another may primarily strengthen patient education or community engagement. These differences should be expected and should be reflected in any FDA framework for understanding PFDD meeting impact. The appropriate measure of success should depend on the needs of the community, the maturity of the evidence base, the development landscape, and the intended use of the patient input.

By communicating impact in this measured way, FDA can strengthen confidence in PFDD without turning impact assessment into an overly rigid exercise. The goal should be a more transparent understanding of how PFDD meetings contribute to patient-centered development and decision-making, not a narrow accounting exercise that undervalues indirect or emerging effects. That measured approach would support accountability, avoid undervaluing indirect or emerging impacts, and keep the focus on practical learning rather than on a narrow accounting of attribution.

Conclusion

The NHC recognizes FDA's continued leadership in advancing PFDD and appreciates the Agency's effort to better understand how PFDD meetings have affected patient communities, research priorities, medical product development, clinical practice, and related stakeholder activities. The NHC views this RFI as a constructive opportunity to make the impact of PFDD meetings more visible and to support more consistent use of patient experience information across the medical product lifecycle. The Agency's continued leadership will help ensure that PFDD meetings remain connected to concrete improvements in research, development, regulatory review, and patient-community capacity. That implementation focus is essential because patient-centeredness depends not only on the collection of patient input, but also in how that input is used.

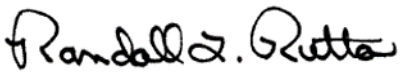
The NHC emphasizes that the value of PFDD meetings depend on how patient input is documented, interpreted, supplemented where appropriate, and used in ways that are meaningful to patients and caregivers. PFDD meetings can help identify unmet needs, clarify what outcomes matter, improve research and development strategies, strengthen patient-community capacity, and support more patient-centered regulatory and clinical conversations. Those impacts should be recognized even when they are not captured in

a single formal regulatory decision. FDA can support those impacts by giving stakeholders clearer expectations for how PFDD outputs should be documented, interpreted, and supplemented.

As FDA moves forward, the NHC urges the Agency to continue centering patients, caregivers, and patient organizations in implementation of PFDD activities. The NHC encourages FDA to use this docket to develop practical tools, examples, and frameworks that help stakeholders understand how PFDD meeting outputs can be used responsibly and effectively. The NHC supports FDA in continuing to build an infrastructure that helps patient input move from engagement into action and welcomes the opportunity for continued dialogue as the Agency considers these comments and advances this work.

Please do not hesitate to contact Kimberly Beer, Senior Vice President, Policy & External Affairs, at kbeer@nhcouncil.org, or Shion Chang, Assistant Vice President, Policy & Regulatory Affairs, at schang@nhcouncil.org, if you or your staff would like to discuss these comments in greater detail.

Sincerely,



Randall L. Rutta
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