Booz Allen

Assessment of the Use of Patient Experience Data in Regulatory Decision-Making

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1. Executive Summary

The U.S. Food and Drug Administration (FDA) views patient experience data, which includes information about how a disease affects a patient's daily life and patient preference for treatment, as an integral component of the drug development and review process. The 21st Century Cures Act (2016), and subsequent reauthorizations of the Prescription Drug User Fee Act (PDUFA), formally directed FDA to advance methods and approaches to capture and measure patients' experiences and perspectives in drug development and review. In accordance with the Cures Act, this report represents the second in a series of three independent assessments (i.e., 2021, 2026, and 2031) that examine FDA's use of patient experience data in regulatory decision-making. For this 2026 report, Booz Allen Hamilton (Booz Allen) conducted interviews with 101 FDA staff who review patient experience data, including clinical and statistical discipline review staff, subject matter experts in qualitative and quantitative analysis of patient experience data (including psychometricians) across FDA's Center for Drug Evaluation and Research (CDER) and the Center for Biologics Evaluation and Research (CBER). Interview participants provided feedback on when FDA reviewers discuss patient experience data in the precompetitive stage and also described how they consider patient experience throughout the drug development and marketing application review; how reviewers communicate FDA's use of patient experience data to sponsors and the public; and challenges and opportunities for improving the use of patient experience data in regulatory decision-making (summarized below).

FDA's Use of Patient Experience Data Across the Development Lifecycle: During early development, FDA reviewers may engage with sponsors prior to Investigational New Drug (IND) submission to align on the types, sources, and collection methods for patient experience data. These discussions can help sponsors design development programs that generate evidence that is meaningful to both patients and regulators. At the premarket phase, patient experience data can inform endpoint selection, clinical trial design, and considerations for the planned benefit-risk assessment. FDA reviewers may use patient experience data to characterize disease burden, establish unmet medical need, and ensure proposed endpoints reflect what patients value. Subject matter experts in qualitative and quantitative analysis of patient experience data (including psychometricians) also consult on whether COAs are fit-for-purpose (i.e., is the proposed assessment appropriate for the target population). During the regulatory review of the marketing application, patient experience data transitions from a tool that can inform and shape product development strategy to evidence evaluated in support of regulatory decisions, including benefit-risk determinations and labeling claims.

Communicating FDA's Use of Patient Experience Data: FDA communicates its use of patient experience data to sponsors through meeting minutes, review documents, Information Requests, and the Patient Experience Data Table. For approved drugs and biologics, FDA publishes redacted review documents on the Agency website as the primary means through which the public can understand how patient experience data was considered. Public-facing communication about patient experience data relies primarily on publishing redacted review documents, issuing draft and final guidance documents, FDA staff authored peer-reviewed publications and participation in conferences.

Challenges in Using Patient Experience Data to Inform Regulatory Decision-Making: FDA reviewers identified several current challenges to the meaningful application of available patient experience data. When assessing data quality and methodological rigor, FDA reviewers expressed that sponsors sometimes submit large volumes of patient experience data without sufficient context

regarding its intended purpose. Moreover, weaknesses in qualitative, quantitative, and mixed-methods research (e.g., unclear saturation methods, inadequate sample sizes, and weak psychometric properties) reduce the methodological rigor of the sponsor-submitted data. Other challenges identified included representativeness where submitted patient experience data may overrepresent highly engaged patients while underrepresenting those with severe disease, geriatric or pediatric populations, and individuals with low health literacy, limiting the generalizability of findings. Additionally, operational limitations stemming from increasing reviewer workloads and staffing constraints can limit FDA attendance at external meetings.

Opportunities for Improving the Use of Patient Experience Data: Sponsors are encouraged to engage FDA before initiating clinical trials and to adhere closely to FDA guidance (e.g., the Patient-Focused Drug Development [PFDD] guidance series) to optimize collection of patient experience data, reduce participant burden, and ensure that COAs are fit-for-purpose. FDA interview participants expressed the importance of detailed meeting requests where sponsors seeking pre-IND or IND meetings should submit clear, thorough meeting requests to enable FDA to identify and engage the appropriate review disciplines and subject matter experts efficiently. At the marketing application review, it is not always clear which review discipline is best positioned to evaluate specific types of patient experience data, highlighting a need for clearer delineation of roles. FDA staff also stated that sponsors are encouraged to include information in the Reviewer's Guide section of the submission that clearly describes the patient experience data submitted and its intended purpose within the review. Participants expressed that an appropriately detailed sponsor-populated Reviewer's Guide reduces the possibility of data being inadvertently overlooked within large application files.

In addition to the findings collected from the qualitative focus group interviews, Booz Allen also assessed a cohort of New Drug Application (NDA) and Biologics License Application (BLA) submissions approved between February 5, 2021, and December 31, 2025, for mentions of patient experience data in reviewer-generated documents and approved labels (herein referred to collectively as “regulatory review documentation”). The quantitative assessment cohort of 800 submissions included all 315 original new molecular entity (NME) NDAs and original BLAs approved within the specified timeframe, 106 original non-NME NDA and BLA submissions, and 379 NDA and BLA efficacy supplements. For this assessment, all NMEs were included for consistency with the 2021 independent assessment. The original non-NME NDA and BLA submissions and efficacy supplements were only included in this assessment cohort if they contained clinical data. In the assessment cohort:

- ~73% of original NME NDA and original BLAs, 60% of original non-NME NDA and BLAs, and 60% of efficacy supplements included mentions of patient experience data (refer to Table 4-2).
- ~13% of all NME submissions in the cohort contained at least one reference to non-sponsor submitted patient experience data such as PFDD meetings, Voice of the Patient Reports, and peer-reviewed literature (refer to Table 4-2).
- ~40% of approved product labels mentioned patient experience data (refer to Table 4-5).

The prevalence of patient experience data within NMEs, non-NMEs, and efficacy supplements differed for special designations and across therapeutic areas.

From the regulatory review documentation analysis, adherence to Section 3001 of the Cures Act was highest for original NMEs at 96%, followed by 84% for non-NME submissions. Adherence was lower for efficacy supplements (66%) and represents an area for investigation and continued focus.

Through the combination of FDA staff focus groups and a thorough assessment of regulatory review documentation, Booz Allen determined that patient experience data is considered and leveraged by FDA throughout the drug development process.

2. Background

PFDD is an approach to help capture and meaningfully incorporate patients' experiences, perspectives, needs, and priorities into the development and evaluation of medical products throughout the medical product lifecycle.[1]

The 2012 PDUFA V reauthorization[2] and the 21st Century Cures Act of 2016 (Cures Act) gave FDA a stronger statutory foundation to further its existing PFDD practices to systematically gather, assess, and incorporate patient perspectives in the drug review process.

The Cures Act, as amended by Section 605 of the FDA Reauthorization Act (FDARA)[3] of 2017, amended the Federal Food, Drug, and Cosmetic (FD&C) Act to define patient experience data as:

> *"(1) data collected by any persons (including patients, family members and caregivers of patients, patient advocacy organizations, disease research foundations, researchers, and drug manufacturers); and*
> *(2) are intended to provide information about patients' experiences with a disease or condition, including*
> > *(A) the impact (including physical and psychosocial impacts) of such disease or condition, or a related therapy, or clinical investigation on patients' lives; and*
> > *(B) patient preferences with respect to treatment of such disease or condition."*

The Cures Act created obligations for FDA, including publishing a brief statement regarding patient experience data submitted and reviewed as part of an approved drug or biologics application (Section 3001), publishing guidance on PFDD (Section 3002), and publishing reports in 2021, 2026, and 2031, describing the Agency's use of patient experience data in regulatory decision-making (Section 3004).

To facilitate compliance with Section 3001 of the Cures Act, FDA developed the Patient Experience Data Table (Figure 3-1), which summarizes the types of patient experience data submitted and reviewed and describes where to find patient experience data in the regulatory review documentation. The review documents published on Drugs@FDA: FDA Approved Drugs[4] for applications approved by CDER and Biological Approvals by Year[5] for applications approved by CBER include the Patient Experience Data Table.

[1] Patient-Focused Drug Development Glossary, FDA
[2] PDUFA Reauthorization Performance Goals and Procedures Fiscal Years 2013 Through 2017
[3] FDA Reauthorization Act of 2017
[4] Drugs@FDA: FDA-Approved Drugs
[5] CBER Biological Approvals by Year

In accordance with Section 3002 of the Cures Act and the PDUFA VI reauthorization,[6] FDA issued a four-part PFDD guidance series that provides industry, FDA staff, and other stakeholders (e.g., patient advocacy organizations) a methodological framework for collecting and submitting patient experience data to FDA in support of medical product development and regulatory decision-making.[7] [8] [9] [10] Recently, in February 2026, FDA also issued and sought input from the public[11] on the E22 General Considerations for Patient Preference Studies draft guidance summarizing considerations on the use, design, conduct, analysis and submission of patient preference studies to inform drug development, and regulatory evaluation.[12]

As required by Section 3004 of the Cures Act, FDA published the first report prepared by a third-party contractor on the Assessment of the Use of Patient Experience Data in Regulatory Decision-Making in June 2021.[13] The 2026 independent assessment, described below, is the second report in the series of three (i.e., 2021, 2026, and 2031).

This report builds on the 2021 report and assesses FDA's use of patient experience data in regulatory decision-making through:

- Collection of feedback from FDA review staff and leadership interviews (summarized in the FDA Review Staff Interviews section) with CDER and CBER clinicians, statisticians, and subject matter experts in qualitative and quantitative analysis of patient experience data (including psychometricians) and;
- Examination of FDA regulatory review documentation for a cohort of NDA and BLA submissions spanning nearly five years, for mentions of patient experience data (summarized in Regulatory Review Documentation Analysis section).

3. Methods

3.1. Focus Group Interviews with FDA Staff

Booz Allen scheduled and hosted 27 focus group interviews, totaling 101 review staff across CDER and CBER. Focus group interview participants came from an assortment of review divisions and roles, including a broad sample of clinical and statistical disciplines and staff who specialize in the review of patient experience data, and subject matter experts in qualitative and quantitative analysis of patient experience data (including psychometricians). Booz Allen developed an interview guide to uniformly collect findings, and obtained staff feedback on: (1) the frequency with which they encounter patient experience data; (2) how patient experience data is considered by reviewers throughout the drug development program (e.g., during early development, premarket phase, marketing [NDA/BLA] application review); (3) how FDA communicates the use of patient experience

[6] PDUFA Reauthorization Performance Goals and Procedures Fiscal Years 2018 Through 2022
[7] FDA Guidance: Patient-Focused Drug Development: Collecting Comprehensive and Representative Input
[8] FDA Guidance: Patient-Focused Drug Development: Methods to Identify What Is Important to Patients
[9] FDA Guidance: Patient-Focused Drug Development: Selecting, Developing, or Modifying Fit-for-Purpose Clinical Outcome Assessments
[10] FDA Guidance: Patient-Focused Drug Development: Incorporating Clinical Outcome Assessments Into Endpoints for Regulatory Decision-Making
[11] FDA-2026-D-0207: E22 General Considerations for Patient Preference Studies; International Council for Harmonisation; Draft Guidance for Industry; Availability
[12] FDA Guidance: E22 General Considerations for Patient Preference Studies
[13] 2021 Assessment of the Use of Patient Experience Data in Regulatory Decision-Making

data to sponsors and the public; and (4) challenges and opportunities for improving the use of patient experience data in regulatory decision-making.

3.2. Assessment Cohort

The assessment cohort described below included a subset of submissions approved between February 5, 2021, and December 31, 2025. The approval cutoff date was chosen to allow sufficient time to collect data and perform the assessment. During this approval timeframe, Booz Allen found 1,463 approved submissions, 1,291 coming from CDER and 172 from CBER and examined:

- All original NME[14] NDAs and original BLAs in the assessment cohort.
- A subset of original non-NME NDAs and BLAs and efficacy supplements (i.e., post-approval submissions to change the approved product label [e.g., to expand treatment to new indications or ages] that requires new clinical evidence). The subset of original non-NME NDAs and BLAs and efficacy supplements was identified by randomly sampling submissions after applying all prespecified inclusion and exclusion criteria (as described below) to promote equitable representation of CDER and CBER offices.

For purposes of the documentation review, Booz Allen included applications receiving final approval under section 351(a) of the Public Health Service Act for BLAs and section 505(c) of the Federal Food, Drug, and Cosmetic Act for NDAs, including both 505(b)(1)[1] and 505(b)(2)[2] applications, when the submission contained clinical data. Booz Allen excluded tentatively approved submissions, submissions that did not contain clinical data, and applications approved under other statutory pathways. To increase the diversity of the cohort and the number of unique products represented, Booz Allen included no more than one efficacy supplement per product. To maximize diversity in the sample and increase the number of unique products represented, Booz Allen also de-duplicated efficacy supplements by including only one supplement per product in the documentation review cohort.

After applying all eligibility criteria, the cohort sample included 1,123 submissions. Booz Allen then sampled ~71% of eligible CDER and CBER submissions to obtain 800 NDA and BLA submissions, including original NMEs, original non-NMEs, and efficacy supplements. The final assessment cohort included roughly proportional numbers of original and supplement submissions, with 315 original NMEs (~39%) across both medical product centers. Table 3-1 summarizes the cohort distribution by application type, submission type, and medical product center.

[14] Original NME: an application that contains a new molecular entity (i.e., an active moiety never previously approved by FDA and is an original, or first submission for that drug or biologic).

Table 3-1: Distribution of the Assessment Cohort by Submission Type and Reviewing Center*

Trait	Categories	Distribution in Submissions (n=800) by %
Application Type	Original NDAs	33.13%
	NME NDAs	*20.63%*
	Non-NME NDAs	*12.50%*
	Original BLAs	19.50%
	NME BLAs	*18.75%*
	Non-NME BLAs	*0.75%*
	Efficacy Supplements	47.38%
	NDAs	*30.13%*
	BLAs	*17.25%*
Review Center	CDER	84.88%
	CBER	15.13%

* NDAs, BLAs, and efficacy supplements approved by FDA between February 5, 2021, and December 31, 2025

3.3. Regulatory Document Review and Analysis

Booz Allen examined the regulatory review documentation in the five-year assessment cohort for mentions of patient experience data based on definitions described in the PFDD Glossary.[15] To promote uniformity in data collection, Booz Allen prospectively determined a set of review documents to be examined (summarized in Table 3-2). The review documents discussed below were selected because they represent the most plausible locations for mentions of patient experience data to reside. Booz Allen accessed the unredacted review documents from FDA's regulatory management and tracking systems to examine for mentions of patient experience data.

Table 3-2: Types of Regulatory Review Documents Assessed for Mention of Patient Experience Data

CDER and CBER Review Documents	

• Primary Clinical Review
• Cross-Discipline Team Lead (CDTL) Summary Memo
• Division Director Summary Memo
• Signatory Authority Decision Memo
• Advisory Committee (AC) Public Meeting Briefing Document
	• Discipline Review Memos
	  ○ Statistical (Biostatistics)
	  ○ Statistical (Patient-Focused Statistical Scientists)
	  ○ COA (Clinical Outcome Assessment)
	  ○ Controlled Substance
	  ○ Labeling
	• Approved Label

CDER-specific Review Documents	**CBER-specific Review Documents**
• Integrated/Multidisciplinary Review* • Discipline Review Memo: Clinical Pharmacology	• Summary Basis of Regulatory Actions (SBRA)^ • Discipline Review Memo: BLA Clinical and Clinical Pharmacology

*Accessed from CDER's regulatory systems | ^Accessed from CBER's regulatory systems

[15] Patient-Focused Drug Development Glossary, FDA

During document reviews, Booz Allen independently examined each of the regulatory review documents summarized in Table 3-2 (above) to locate the Patient Experience Data Table (illustrated in Figure 3-1) or the Patient Experience Data Section and searched for the mention of patient experience data throughout the respective document. Upon identifying the mention of patient experience data, Booz Allen recorded the location and types of patient experience data referenced (derived from those listed in the Patient Experience Data Table, pictured below).

Patient Experience Data Relevant to this Application (check all that apply)

1	☐	The patient experience data that were submitted as part of the application include:		Section of review where discussed, if applicable
2		☐	Clinical outcome assessment (COA) data, such as	
3			☐ Patient reported outcome (PRO)	
4			☐ Observer reported outcome (ObsRO)	
5			☐ Clinician reported outcome (ClinRO)	
6			☐ Performance outcome (PerfO)	
7		☐	Qualitative studies (e.g., individual patient/caregiver interviews, focus group interviews, expert interviews, Delphi Panel, etc.)	
8		☐	Patient-focused drug development or other stakeholder meeting summary reports	
9		☐	Observational survey studies designed to capture patient experience data	
10		☐	Natural history studies	
11		☐	Patient preference studies (e.g., submitted studies or scientific publications)	
12		☐	Other: (Please specify):	
13	☐	Patient experience data that were not submitted in the application, but were considered in this review:		
14		☐	Input informed from participation in meetings with patient stakeholders	
15		☐	Patient-focused drug development or other stakeholder meeting summary reports	
16		☐	Observational survey studies designed to capture patient experience data	
17		☐	Other: (Please specify):	
18	☐	Patient experience data was not submitted as part of this application.		

Figure 3-1: The Patient Experience Data Table Template Seen in FDA Review Documentation

4. Assessment Results

4.1. FDA Review Staff Interviews

Through the facilitation of focus group interviews with FDA staff, Booz Allen found that FDA reviewers consider and use patient experience data throughout drug development and the review process. The subsections below describe how patient experience data can inform reviewer thinking and discussions between the sponsor and FDA in early development, prior to the submission of an NDA/BLA, and during a marketing application review. The mutual benefit of early engagement was an overarching sentiment heard throughout FDA staff interviews: FDA staff emphasized that early discussions benefit both sponsors and FDA, as sponsors gain clarity on what evidence will be considered meaningful in support of their program, and FDA reviewers gain a deeper understanding of the therapeutic context and the patient experience underlying the sponsor's development program.

4.1.1. Use of Patient Experience Data in the Precompetitive Stage and Premarket Phases

Precompetitive Stage

FDA's consideration of patient experience data begins even before a sponsor initiates a formal drug development program, with precompetitive forums offering early opportunities for scientific exchange between FDA and the broader research community. During focus group interviews, some CDER reviewers described Critical Path Innovation Meetings (CPIM)[16] [17] as a setting where scientific dialogue between FDA and representatives from patient advocacy organizations, scientific consortia, academia, and industry occurs, particularly about data related to the development of COAs.

Premarket Phase

While available precompetitive forums help establish a shared scientific foundation, the premarket phase marks the point at which patient experience data discussions become more directly tied to a specific sponsor's development program and regulatory strategy. Reviewers consistently emphasized the value of discussions between sponsors and FDA that focus on prospective planning for the collection of patient experience data. Engagement supports sponsor and FDA alignment and can include discussions about what types of patient experience data to collect, from whom (i.e. defining the target population and determining who will provide the patient experience data), and the proposed sampling and data collection methods.

Early stage discussions of patient experience data with FDA include conversations about the appropriateness of planned qualitative research methods, how patient experience data will inform the sponsor's target product profile, and early endpoint planning including the feasibility of collecting data in the intended population (e.g., patient preference studies which can inform endpoint selection or hierarchy, where the sponsor may choose to conduct a study to gather information on ranking outcomes). FDA staff described incremental improvements in sponsor-proposed methodology for collecting patient preference data. As part of the early stage discussions, some reviewers observed that on limited occasions, such discussions occur during INitial Targeted Engagement for Regulatory Advice on CBER/CDER ProducTs (INTERACT) meetings.

When Booz Allen asked reviewers to describe use of patient experience data during the IND phase, FDA staff stated that patient experience data primarily informs four areas of development program discussions between FDA and sponsors: (1) endpoint selection, (2) clinical trial design, (3) COA instrument selection and development and; (4) considerations for the planned benefit-risk assessment. Frequently cited examples of patient experience data considered by FDA in the premarket phase are summarized in Table 4-1.

[16] CPIMs provide non-regulatory, drug-product-independent, nonbinding, scientific advice-seeking discussions between CDER and external stakeholders

[17] Critical Path Innovation Meetings (CPIM), FDA

Table 4-1: Examples of Patient Experience Data Considered by FDA During the Pre-IND and IND Phases as Reported through Focus Group Interviews

Examples of Patient Experience Data Submitted by Sponsors	Additional Sources of Patient Experience Data

• Locations Where FDA Encounters and Considers Patient Experience Data:
  ○ Briefing packages for formal meeting requests
  ○ Documents submitted to the IND outside of formal meeting requests
  ○ Study protocols that include COAs or patient preference instruments
  ○ Proposals for digital health technology use
• Types of Patient Experience Data within:
  ○ Natural history studies
  ○ Focus group materials
  ○ Salient literature
  ○ Patient preference information (PPI)
  ○ Qualitative study reports (concept elicitation and/or cognitive interviews)
  ○ Preliminary psychometric data (e.g., from Phase II trials)
	• Forums Where FDA has Found Additional Patient Experience Data:
	  ○ PFDD Public Meetings (both FDA- and externally-led) for their therapeutic and/or the disease state within their focus area
	  ○ Patient Listening Sessions (both FDA-requested and patient-led)
	  ○ Meeting summary materials
	  ○ Condition-Specific Meeting Reports and other information related to patients' experience
	  ○ Ad hoc literature searches

In addition to sponsor-submitted patient experience data, many reviewers described actively seeking supplemental sources (described on the right panel of Table 4-1) to deepen their understanding of a particular therapeutic context. Participants from both product centers and all review disciplines reported finding great value in Voice of the Patient reports, patient preference studies (PPS), FDA-requested and patient-led listening sessions, and externally-led and FDA-led PFDD public meetings.

Although patient listening sessions and PFDD meetings do not convene in connection with the review of any specific marketing application, reviewers of both clinical and statistical disciplines attend these meetings to build their understanding of the patients' experiences with a condition and their experiences with currently available treatments directly from patient and caregivers. Reviewers added that they routinely review meeting summaries and condition-specific reports as useful resources to augment their understanding of the burden of living with a condition and the limitations of the current standard of care.

Figure 4-1 illustrates the meeting types prior to the submission of a marketing application, where FDA reviewers reported discussing or using patient experience data.

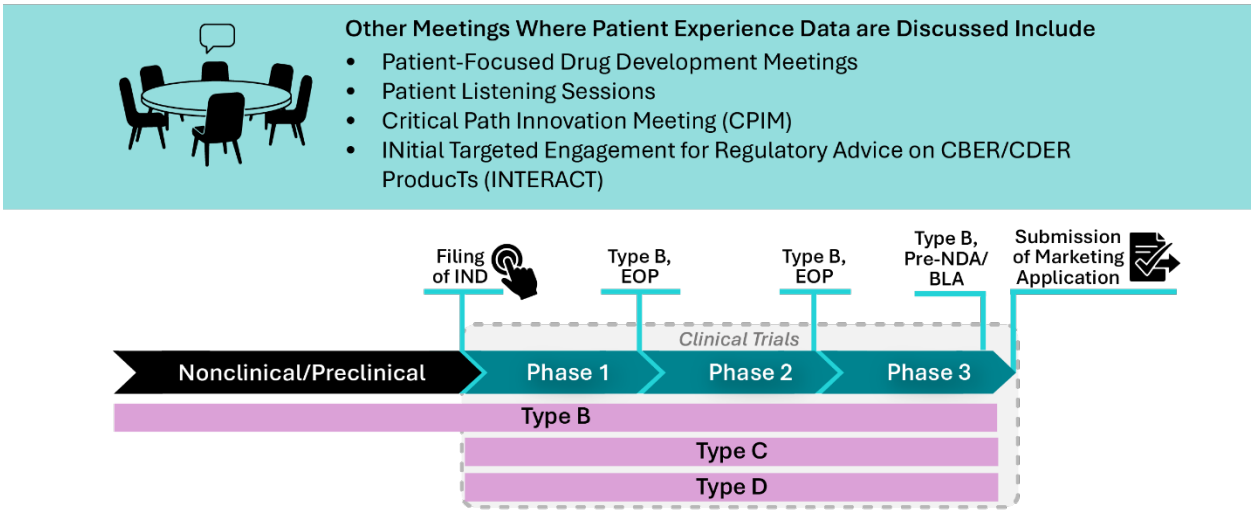

Figure 4-1: Meetings where FDA Reviewers Discuss or Encounter Patient Experience Data[18]

In addition to the purposes and sources of patient experience discussed above, Booz Allen observed that the use of patient experience data during the IND phase is multidisciplinary:

- Clinical reviewers described using patient experience data to help characterize the disease and its burden, understand areas where patients have identified an unmet medical need, and inform clinical protocol development in ways to reduce participant burden, such as reducing the invasiveness of assessments and frequency of data collection.
- Statistical reviewers described reviewing patient experience data collection plans within sponsor-proposed statistical analysis plans, including endpoint hierarchy, meaningful within-patient change thresholds, sample size implications, and how sponsors plan to handle missing data for COA-based endpoints.
- Subject matter experts (SME) in qualitative and quantitative analysis of patient experience data consult with clinical and statistical staff on the review team. The SMEs described using patient experience data to assess the adequacy of PPS and observational study designs, and whether outcome measures are fit-for-purpose. SME assessment includes whether the concepts measured by survey instruments and/or COAs (i.e., Patient-Reported Outcomes [PRO], Clinician-Reported Outcomes [ClinRO], Observer-Reported Outcomes [ObsRO], or Performance Outcomes [PerfO]) are meaningful to patients; assessment of evidence to support the psychometric properties of the instruments including

Role of Sponsor-Submitted Qualitative Research Methods in COA Development
• Concept elicitation data helps FDA assess whether the COA measures what is most important to patients and whether the timeframe for the endpoint is appropriate. • Cognitive interviewing data helps FDA assess whether the wording, recall period, and response options are appropriate and interpretable to patients. • Data collected from patients with different phenotypes or disease presentations also enables FDA to assess whether the COA performs consistently across the range of patients who may be enrolled in the trial.

[18] Chevron sizes are illustrative only, and do not reflect expected duration of phase.

construct and content validity, reliability of the instrument to yield reproducible/consistent results, and the sensitivity of the instrument's ability to detect change.

Patient experience data informs discussions about which concepts to target for measurement, such as symptoms, functioning, and health-related quality of life, and whether those concepts are salient and meaningful to patients. These discussions help sponsors focus their measurement strategy on what matters most to patients while giving reviewers confidence that the proposed endpoints reflect the patient experience of the condition.

Patient experience data further informs discussions about clinical trial design and conduct, including dosing regimens, routes of administration, and trial design features that affect patient burden, such as the frequency and method of data collection/visit schedules, sample size, target patient population, and trial duration. Input from patients on what they consider tolerable and acceptable can help sponsors design trials that work to minimize burden to patients while generating meaningful and scientifically robust evidence.

In addition, patient experience data is central to initial discussions about benefit-risk. Early signals from patient experience data can inform both sponsors and reviewers about whether the benefit-risk profile of a treatment is likely to be acceptable to patients, including an understanding of what side effects or burdens patients consider tolerable given the severity of their condition. FDA reviewers noted this consideration as particularly relevant in oncology and rare disease settings, where patients may have a higher tolerance of risk in exchange for potential benefit.

4.1.2. Use of Patient Experience Data in the NDA/BLA Regulatory Review

When asked to describe the types of sponsor-submitted patient experience data that FDA staff considers during the review of a marketing application, a majority of participants observed that many of the sources and types of patient experience data reviewed during the NDA/BLA review are similar to those used during the IND phase (see Table 4-1). FDA staff stated that between the premarket and NDA/BLA regulatory review, the intended purpose and rigor of the evaluation differs. Review staff from both centers shared that during the IND phase, patient experience data primarily serves to shape clinical trial strategy and build reviewer understanding of the patients' experiences with the disease state and what matters most to patients. At the NDA/BLA regulatory review, patient experience data collected as part of the development program is evaluated as evidence to inform regulatory decisions. During focus group interviews, FDA participants expressed sentiment that patient experience data can and does inform the benefit-risk assessment and labeling claims during the review of the marketing application.

Focus group interview participants observed that sponsor inclusion of COAs in submissions across therapeutic areas has increased, and reviewers described a similar upward trend in uses of COAs as secondary endpoints for chronic conditions, particularly in aging populations, oncology, and obesity. Participants observed that in some therapeutic areas, PROs provide additional information on tolerability of the product.

During the NDA/BLA review, FDA staff participants stated that they use patient experience data to assess whether the overall benefit-risk profile of a product remains favorable for the intended patient population. When assessing the benefit-risk profile, clinical reviewers consider patient input by drawing on sources such as PFDD meeting summaries, patient advocacy group surveys, and natural history studies to provide broader context for the patient experience. In development programs where a COA is used as a primary or key secondary endpoint, or where a sponsor is seeking COA-based labeling claims, reviewers stated they conduct a thorough evaluation of the instrument's

development history, psychometric properties, and the clinical meaningfulness of the observed treatment effect. This evaluation requires specialized expertise, and staff across clinical and statistical disciplines, including those who specialize in the review of patient experience data, SMEs in qualitative and quantitative analysis of patient experience data (including psychometricians), collaborate in the analysis and interpretation of the patient experience data that supports and contextualizes the benefit-risk analysis.

Reviewers noted that while COA data collected as part of a clinical trial is generally straightforward to locate within an application, locating other types of patient experience data presents a greater challenge. FDA added language in the eCTD Technical Conformance Guide[19] Technical Specifications document in 2019. The language asks sponsors to provide information using a table very similar to FDA's Patient Experience Data Table in the Reviewer's Guide. FDA staff reported that few sponsors are currently including the guiding information in their applications. Interview participants explained that applications routinely contain thousands of pages within files, and without the sponsor identifying the patient experience data submitted, its location within the application, and how the sponsor intends for it to inform the review, relevant information can be inadvertently overlooked.

eCTD Components Where Patient Experience Data Has Been Located
Module 1: Administrative Information: • Formal meetings • Cover letters • Dispute resolutions • Information amendments • Requests for comment and advice • Draft labeling • Reviewer's Guide
Module 2: Summaries: • Clinical overviews • Clinical summaries (e.g., summary of clinical pharmacology studies)
Module 5: Clinical Study Reports: • Reports of efficacy and safety studies • Reports of human pharmacokinetic studies

Reviewers noted that patient experience data may be submitted across multiple sections of an application. The insert above lists the numerous locations where patient experience data is found within the eCTD.

4.1.3. Communicating FDA's Use of Patient Experience Data

When asked how FDA communicates its use of patient experience data, reviewers described using several methods. They also drew a clear distinction between two types of communication: product-specific communication, explaining how patient experience data was used in the review of a particular medical product; and communications where FDA shares its current thinking and approaches to using patient experience data across products more broadly.

Throughout the interviews, reviewers described communicating their use of patient experience data to sponsors across the review process, beginning as early as the first formal interaction between a sponsor and FDA. During the IND phase, meeting minutes provided to sponsors following formal interactions with FDA capture discussions on the use of patient experience data. During the NDA/BLA review, discussion of patient experience data is typically found in the relevant section of the review document, and the Patient Experience Data Table captures a range of patient experience data sources considered during the review, including COAs, PFDD meeting summaries, and PPS.

[19] eCTD Technical Conformance Guide

Reviewers noted that the discussion of patient experience data that serves as an endpoint tends to be more explicit and detailed than the discussion of patient experience data that is intended to inform the reviewer's understanding of the therapeutic context. Reviewers acknowledged that stakeholders have expressed interest in more detailed information about how patient experience data is considered in public-facing review documentation. However, review documentation focuses primarily on the evidence submitted as part of the marketing application, which may not fully reflect the role that patient experience data played in informing discussions and decisions throughout the IND phase. This may contribute to the impression among some stakeholders that patient experience data does not get considered until the marketing application stage, when in fact it informed reviewer thinking and sponsor-FDA discussions throughout the development program.

For the NDA/BLA review specifically, participants across both product centers identified the authorship of review documents and when appropriate, the approved label as the most prominent and visible means of communicating their use of patient experience data. Interview participants also identified the summary review document used to draw connections between the interpretations and conclusions of different review disciplines as another means of communicating. In addition, review staff stated that when FDA issues an Information Request seeking additional patient experience data or clarification about patient experience data that has already been submitted, sponsors gain further insight into how FDA is considering patient experience data as part of the review. The public-facing record that has review documents for approved drugs and biologics and is published on FDA's website represents the primary means through which the public can understand how patient experience data was considered.

Beyond the review process itself, FDA communicates its approach to patient experience data through a range of broader public-facing channels. FDA communicates its current thinking on the use of patient experience data through the issuance of draft and final guidance documents and the publication of articles in peer-reviewed journals. FDA staff also engage with the public through speaking engagements at scientific conferences, academic research institutions, professional organization events, and disease-specific organization workshops, though reviewers noted that staffing and funding constraints can affect the frequency of FDA attendance at these engagements.

During focus group interviews FDA staff elaborated that meetings with patient groups are a means through which the Agency broadly communicates how it considers patient experience data. However, interview participants emphasized that their ability to discuss patient experience data specific to a development program is limited. Review staff reiterated that FDA has statutory obligations[20] [21] that it must comply with to protect confidential commercial information and trade secrets. Review staff stated that when speaking to anyone other than the sponsor, FDA generally cannot acknowledge the existence of a specific application under review and can only discuss the disease area and general patient experience.

FDA interviewees noted that they contribute general COA-related recommendations to public-private partnerships. FDA also communicates its use of patient experience data through FDA.gov, including via the Drug Development Qualification Programs (e.g., CDER and CBER's Drug Development Tool [DDT] Qualification Project Search database) and through the Oncology Center of

[20] 21 U.S.C. § 331(j)
[21] 21 CFR Part 20

Excellence's PFDD Project Patient Voice[22], which provides patients, caregivers, and healthcare providers access to the labels of approved products that reference PROs.

4.1.4. Challenges in the Use of Patient Experience Data in Regulatory Decision-Making

When asked to identify and describe challenges FDA experiences when using patient experience data to support the regulatory review process, interview participants described challenges across three categories: (1) data quality and methodological rigor; (2) representativeness and interpreting meaningful change; and (3) operational limitations.

Data and Methodological Quality

With respect to data quality, interview participants observed an anecdotal and incremental increase in quality of patient experience data received from sponsors. However, they also stated that some challenges persist. Reviewers observed that when sponsors delay engagement with FDA until the submission of the marketing application, they miss the opportunity to align FDA and sponsor expectations regarding how the types of patient experience data included in the application should inform the review.

FDA staff noted they assess data quality before beginning analysis, and insufficient or missing data and information makes it difficult for FDA to determine the credibility of the source and whether the data collection methodology was rigorous. The multidisciplinary review teams identified several issues they encounter when reviewing both qualitative and quantitative research. For example, unclear saturation methods, poor interview design, and/or insufficient transparency in data collection methods are issues that have been seen in qualitative research, and while reviewing some quantitative research, insufficient statistical power/sample size, inappropriate statistical methods, inadequate prospective planning and handling of missing data, and weak psychometric properties have been observed. A typical challenge noted by reviewers involves situations in which the Agency provided advice on qualitative research (including specific interview questions), but sponsors subsequently do not submit their interview guides, making it difficult to assess whether the recommended questions were actually incorporated into the research. Reviewers also stated that limited, missing, and improperly formatted data (e.g. not using appropriate data standards) can result in time-consuming Information Requests (IR) that create additional work for sponsors and decrease the FDA team's active review time.

Representativeness and Interpreting Meaningful Change

During marketing application review, FDA evaluates whether the submitted patient experience data reflects the target population. They report that on occasion they encounter underrepresentation of important sub-populations such as geriatric and pediatric patients, those with severe disease, and populations with low health literacy. This can lead to a nonrepresentative sample, which in turn may lead to non-generalizable conclusions for the treatment population. Pertaining to interpreting meaningful changes, when FDA is examining what constitutes meaningful within-patient change, participants cited a lack of established responder thresholds as a challenge they sometimes face.

Operational Limitations

[22] Project Patient Voice, FDA

Using patient experience data throughout the regulatory review processes requires a deeply collaborative and multidisciplinary approach from FDA. During focus group interviews with FDA, reviewers noted that it is not always clear which review discipline is best positioned to evaluate specific types of patient experience data, particularly as the range of data types submitted by sponsors continues to expand. Reviewers identified clarifying roles, responsibilities, and best practices of when to engage specialized expertise across disciplines as areas for potential improvement.

During focus group interviews, FDA reviewers of both clinical and statistical disciplines in CDER and CBER described attending listening sessions and PFDD meetings to build their understanding of the patient and caregiver experience with the condition and with existing treatments (independent from the review of any specific marketing application). However, despite high interest in attending these engagements, increasing reviewer workload and staffing limitations in some cases preclude their attendance. In situations where workload limits engagement, reviewers stated they routinely review meeting summaries and condition-specific reports as useful resources to augment their understanding of the burden of the condition and the limitations of the current standard of care.

4.1.5. Opportunities for Improving the Use of Patient Experience Data in Regulatory Decision-Making

Early Product Development

Reviewers universally mentioned the importance of early sponsor engagement (i.e., before beginning clinical trials) and close adherence to FDA guidance, particularly the PFDD guidance series. This increases the likelihood of achieving the following positive outcomes in the sponsor's development program:

- Characterization of the disease (e.g., via natural history data and patient listening sessions)
- Assessment of health concepts that are meaningful to patients
- Optimization of plans for collecting patient experience data (e.g., reducing burden on participants, promoting higher participant completion rates, mitigating potential missing data issues and sources of bias such as attrition bias)
- Use of COAs that are fit-for-purpose

Premarket Phase

Reviewers also encouraged sponsors seeking pre-IND or IND meetings to provide clear and detailed meeting requests so that FDA has the necessary information to properly identify and invite the appropriate review disciplines and SMEs. Thorough sponsor meeting requests allow FDA to maximize the use of limited reviewer resources and improve ability to address sponsor questions and provide relevant and consistent guidance for the specific development program (e.g., appropriate statistical and psychometric analysis to inform regulatory decision-making), reducing scenarios where FDA experts are consulted after the completion of a sponsor's pivotal trials.

Use of Patient Experience Data in the NDA/BLA Regulatory Review

Based on findings from FDA staff interviews, and increasing complexity of patient experience data, sponsors are encouraged to provide details in the Reviewer's Guide describing what patient experience data has been submitted, where it is located and how the sponsor intends for such data to inform the NDA/BLA review.

4.2. Regulatory Review Documentation Analysis

Booz Allen examined a cohort of 800 NDA, BLA, and efficacy supplement reviews approved between February 5, 2021, and December 31, 2025 (see Section 3.2 for details on sampling methodology). The cohort included original NME NDA and BLA submissions[23], original non-NME NDA and BLA submissions, and efficacy supplements. Consistent with the approach taken in the 2021 Assessment of the Use of Patient Experience Data in Regulatory Decision-Making[24], the 2026 document analysis focused on original NME submissions as an effective proxy for submissions most likely to contain references to patient experience data, though general trends for original non-NMEs and efficacy supplements are described where informative.

A majority of FDA reviews within the cohort, 65%, referenced patient experience data. Reviews that did not reference patient experience data may reflect a variety of circumstances. For example, a subset of submissions included in the cohort relate to biological products subject to licensure under Section 351(a) of the Public Health Service (PHS) Act that also meet the definition of "device" in Section 201(h) of the FD&C Act. Although subject to licensure, these devices are used in the manufacture of blood and blood components, including screening donated blood and blood components and human cells, tissues, and cellular and tissue-based products for infectious diseases and for related blood banking practices, such as blood typing and compatibility testing to ensure safe transfusion. Therefore, patient experience data is not applicable during the review of these devices.

4.2.1. Capturing Patient Experience Data in Reviewer-Generated Documentation

Booz Allen found mentions of patient experience data used to support the review of drug and biologic applications in the Patient Experience Data Table, and elsewhere across the regulatory review documentation.

Among original NME submissions, 73% of reviews referenced patient experience data, a higher percentage than observed for both original non-NME submissions and efficacy supplements (refer to Table 4-2). This finding is consistent with the 2021 assessment which found that original NME submissions contained patient experience data at higher rates than other submission types.

Table 4-2: Mentions of Patient Experience Data in FDA Reviews of Approved NDAs and BLAs (n=800), Stratified by Submission Type and Source

Metric	Original NME (n=315)	Original Non-NME (n=106)	Efficacy Supplement (n=379)
Percent of approved submissions* where review documents mention patient experience data	73.02%	60.38%	60.42%
Percent of approved submissions where review documents mention patient experience data **submitted by sponsors**	68.57%	60.38%	57.78%

[23] For this analysis, all original BLAs in the cohort are considered NMEs.
[24] https://www.fda.gov/media/150405/download?attachment

Metric	Original NME (n=315)	Original Non-NME (n=106)	Efficacy Supplement (n=379)
Percent of approved submissions where review documents mention patient experience data from **other sources**	12.70%	5.66%	4.49%

*The 2021 independent assessment reported on approved applications where review documents mention patient experience data. Because this report also includes an assessment of efficacy supplements the term "submission" is used.

References to patient experience data in NME reviews increased over time within the assessment cohort, from 71% in 2021 to 85% in 2025. Table 4-3 summarizes the prevalence of patient experience data mentions in original NME submissions stratified by special considerations. Among the noteworthy findings, approved applications granted orphan drug status had the highest percentage of reviews mentioning patient experience data at 91.5%. Reviewers noted during focus group interviews that they frequently encounter patient experience data in submissions for rare diseases, a finding that is consistent with this quantitative result. Reviewers suggested that this may be due to rare disease submissions often representing one of the first proposed treatments for a condition where there is limited published literature on endpoints, natural history, and other clinically relevant information. In this context, patient experience data plays a particularly important role in characterizing the disease, establishing what matters most to patients, and supporting the development of fit-for-purpose endpoints.

Table 4-3 illustrates the prevalence of patient experience data mentions in regulatory review documentation for original NME submissions, stratified by special considerations.

Table 4-3: Mentions of Patient Experience Data in FDA Reviews of FDA Reviews of Approved Original NME NDAs and BLAs (n=315), Stratified by Special Feature and Source

Metric	Standard (n=124)	Priority Review (n=180)*	Orphan Drug (n=153)
Percent of approved applications where review documents mention patient experience data	68.55%	80.56%	91.50%
Percent of approved applications where review documents mention patient experience data from **the sponsor**	62.90%	76.67%	88.24%
Percent of approved applications where review documents mention patient experience data from **other sources**	16.13%	11.11%	16.99%

Note that the number of standard NME submissions and priority review NME submissions do not add up to the total number of NME submissions (315) because there are 11 submissions for devices used in the manufacture of blood and blood components for which patient experience data is not applicable.*

4.2.2. Types of Patient Experience Data Documented in Reviewer-Generated Documentation

Among FDA reviews of original NME submissions that mention patient experience data, PROs were the most frequently cited type of COA, consistent with the findings of the 2021 assessment and sentiments conveyed by FDA staff during the 2026 focus group interviews. The prevalence of PROs in reviewer-generated documentation remained relatively stable between the two assessment periods, at 84% in the 2021 cohort and 80% in the 2026 cohort.

Modest increases in the prevalence of ClinROs, ObsROs, and PerfOs were observed compared to the 2021 cohort. The reasons for these increases cannot be determined from this assessment alone, as many factors influence the types of patient experience data submitted and reviewed over time, including the evolution of the science, the maturation of fit-for-purpose tools, and changes in sponsor practices. It is also worth noting that the PFDD guidance series, which provides a methodological framework for collecting and submitting patient experience data, is still being finalized. The first guidance document in the series was finalized in June 2020 and given that drug development programs and clinical trials span many years, the full effect of the guidance series on sponsor submissions is unlikely to be fully reflected in this or even the next assessment cohort. The 2031 assessment will provide additional insight into whether these trends continue over time.

Among FDA reviews that contain mentions of patient experience data, reviewers more frequently referenced patient experience data submitted by sponsors than data from other sources. However, reviewers also documented having considered patient experience data from sources outside the application in 17% of original NME reviews, compared to 11% in the 2021 cohort. This increase is consistent with the focus group findings, where reviewers described actively seeking supplemental sources of patient experience data, including meeting summaries from patient listening sessions, Voice of the Patient reports, and peer-reviewed literature, to build their understanding of what matters to patients, as well as to better understand the patient and caregiver experience with the condition and with existing treatments. See Table 4-4 for more details.

Table 4-4: Types of Patient Experience Data Mentioned in FDA Reviews of Original NME Submissions, Stratified by the Source

Metric	FDA Reviews that Contained Mentions of Patient Experience Data for Approved Original NME NDAs and BLAs (n = 230)
Of FDA reviews that mention patient experience data, percent that mention data from **sponsors**	93.91%
PRO	80.43%
ClinRO	38.26%
PerfO	11.30%
ObsRO	8.70%
Qualitative studies	7.39%
Natural history studies	4.78%

Metric	FDA Reviews that Contained Mentions of Patient Experience Data for Approved Original NME NDAs and BLAs (n = 230)
PFDD or other stakeholder meeting summaries	2.17%
Patient preference study	1.30%
Of FDA reviews that mention patient experience data, percent that mention data from **other sources**	17.39%
PFDD or other stakeholder meeting summaries	10.87%
Natural history studies	0.43%
Qualitative studies	1.74%

4.2.3. FDA's Use of the Patient Experience Data Table

In accordance with Section 3001 of the 21st Century Cures Act, FDA has a requirement to make a brief statement in review documentation regarding the patient experience data submitted and reviewed as part of an approved drug or biologic application. This requirement is typically fulfilled through the inclusion of a Patient Experience Data Table within the review documentation.

Among the 230 original NME submissions that mentioned patient experience data, 96% of FDA reviews were compliant with Section 3001 of the Cures Act. The vast majority of these instances were compliant via a populated Patient Experience Data Table. This represents a high level of adherence to the statutory requirement and reflects the consistent inclusion of this documentation across NME reviews. A similarly high level of adherence was observed among original non-NME submissions (84%).

Adherence was lower among efficacy supplements (66%). Additional investigation is recommended to identify the root cause of this finding. Improving adherence to Section 3001 across efficacy supplements represents an area for continued focus.

References to Patient Experience Data in the Approved Product Label

Approved product labels in the assessment cohort were examined for the mention of patient experience data. Of the 315 original NMEs in the cohort, 300 had accessible approved product labels. The 15 original BLAs without posted product labels were for blood components intended for further manufacturing use and devices used in the manufacturing of blood and blood components, for which posting would not be expected.

Among the 230 reviews of original NMEs that discuss patient experience data and have an accessible approved product label, approximately 40% of the labels mention patient experience data of any type (Table 4-5). This is an increase from what was observed in the 2021 assessment (30%), suggesting that the prevalence of patient experience data mentions in approved labeling has increased across the two assessment periods. As with the findings in Section 4.2.1, approved applications granted orphan drug status and those receiving priority review had higher rates of patient experience data mentions in approved labeling than the overall cohort, consistent with the

qualitative finding that patient experience data plays a particularly important role in rare disease and other high unmet medical need settings. Prevalence was similar across original non-NMEs and efficacy supplements.

Table 4-5 below outlines trends within the approved labels among the 91 original NME submissions with approved labels referencing patient experience data.

Table 4-5: Prevalence of Patient Experience Data in Original NME Approved Labels, Stratified by Special Feature and Therapeutic Area

Metric	Product Labeling for Approved NME NDAs and BLAs with FDA Reviews that Mention Patient Experience Data (n=91)
Percent of approved product labeling mentioning patient experience data*	39.57%
Percent of approved product labeling mentioning patient experience data by Special Consideration**	
Standard Review	39.56%
Priority Review	60.44%
Orphan Drug	53.85%
Percent of approved product labeling mentioning patient experience data by Therapeutic Area**	
Neoplasms benign, malignant and unspecified (including cysts and polyps)	35.16%
Congenital, familial and genetic disorders	14.29%
Skin and subcutaneous tissue disorders	12.09%
Nervous system disorders	10.99%

*Following the methodology used in the 2021 Report, Booz Allen used the total number of original NME reviews that mention patient experience data (n=230) as the denominator when calculating this percentage.
**Booz Allen used the number of approved product labels containing mention of patient experience data (n=91) as the denominator when calculating percentages for the Special Considerations and Therapeutic Area subsections of the table.

5. Conclusion

Through qualitative and quantitative methods, including focus group interviews with CDER and CBER review staff and examination of FDA review documents and product labeling for a cohort of 800 NDAs, BLAs, and efficacy supplements approved between February 5, 2021, and December 31, 2025, assessment findings determined that FDA considers patient experience data before and throughout the drug development and regulatory review process. The findings of this assessment build on those of the 2021 assessment and reflect continued progress in the integration of patient experience data into regulatory decision-making. Reviewers described using patient experience data across a range of settings and interactions, from precompetitive forums focused on measurement science with the broader scientific community, to formal meetings with sponsors during the IND phase and through the regulatory review of marketing applications. During the IND phase, patient experience data primarily informs four areas of discussion between FDA and sponsors including

endpoint selection, clinical trial design, COA instrument selection and development, and considerations for the planned benefit-risk assessment.

Reviewers emphasized that early engagement between sponsors and FDA centered on patient experience data includes discussions about types of patient experience data to collect (including PPS), and from whom. FDA staff also suggested that maximizing the impact of patient experience data requires sponsor collaboration through methodologically rigorous data collection, clear communication of data purpose and collection methods. In addition, because the review of patient experience data is collaborative and multidisciplinary, with clinical and statistical reviewers, and SMEs in qualitative and quantitative analysis of patient experience data (including psychometricians) each contributing distinct expertise, Booz Allen recommends continued investment in multidisciplinary review capacity.

The quantitative findings from the document review are consistent with the qualitative findings from the focus group interviews. The prevalence of patient experience data in NME reviews increased from 68% in the 2021 assessment cohort to 73% in the 2026 assessment cohort. Within the 2026 cohort, references to patient experience data in NME reviews increased over time, from 69% in 2021 to 85% in 2025, suggesting a continued upward trend in the inclusion of patient experience data in drug development programs and regulatory submissions. Approved applications granted orphan drug status had the highest prevalence of patient experience data mentions in reviews at 91.5%, consistent with the qualitative finding that patient experience data plays a particularly important role in rare disease settings where there is limited published literature on endpoints, natural history, and other clinically relevant information. The prevalence of patient experience data mentions in approved labeling increased to 40% when compared to the 30% observed in the 2021 assessment. As noted throughout this report, the prevalence of patient experience data in approved labeling reflects only a subset of the ways in which patient experience data informed the overall review and should not be interpreted as a comprehensive measure of FDA's use of patient experience data in regulatory decision-making. It should be noted that unlike the 2021 assessment, the 2026 assessment was limited to interviews with FDA review staff due to administrative and resource constraints. The perspectives of sponsors, patients, caregivers, and other stakeholders on FDA's use of patient experience data were not captured in this assessment cycle and represent an important dimension that FDA has conveyed that they hope to once again be able to incorporate in the 2031 assessment.

This assessment also identified several areas for continued focus. Reviewers unequivocally encouraged sponsors to engage with FDA early in the development process and consult the PFDD guidance series. Reviewers also encouraged sponsors seeking pre-IND or IND meetings to provide detailed meeting requests so that FDA can properly engage the appropriate review disciplines and SMEs.

Identifying patient experience data within a marketing application can be challenging given the volume of files and insufficient context of what has been submitted and how it is intended to inform the review. While FDA has addressed this in the eCTD Technical Conformance Guide Technical Specifications document, improving sponsor adherence to this request represents an important opportunity to enhance the efficiency and completeness of the review of patient experience data. Reviewers observed that it is not always clear which review discipline is responsible for evaluating specific types of patient experience data, particularly as the range of data types submitted by sponsors continues to expand and suggested clarifying roles and responsibilities across disciplines as an area for potential improvement. Furthermore, adherence to Section 3001 of the Cures Act was

lower for efficacy supplements (66%) than for original NMEs (96%) and non-NME submissions (84%), which represents an area for investigation and continued focus.

Finally, reviewers acknowledged that current review documentation may not fully reflect the role that patient experience data played during the IND phase, which may contribute to the impression among some stakeholders that patient experience data is only considered at the marketing application stage.

When sponsors engage FDA early and submit patient experience data that is well-organized, clearly documented, and accompanied by a clear description of its intended purpose within the application, the resulting development program is more likely to generate evidence that is meaningful to patients and informative to FDA in its regulatory decision-making.

Appendix A. Patient Experience Data in Review Documentation by Therapeutic Area

Table 5-1 provides the number and prevalence of NME submissions in the assessment cohort with mentions of patient experience data by therapeutic area. Therapeutic area was determined using Medical Dictionary for Regulatory Activities (MedDRA) Hierarchy. MedDRA organizes terms from specific indications through System Organ Classes (SOC), which function similarly to therapeutic areas.

Table 5-1: Prevalence of mentions of patient experience data in the assessment cohort of original NMEs by therapeutic area

Therapeutic Area (MedDRA SOC)	Reviews of Original NMEs that Mention Patient Experience Data
Blood and lymphatic system disorders n=13	8 (62%)
Cardiac disorders n=7	6 (86%)
Congenital, familial and genetic disorders n=43	39 (91%)
Ear and labyrinth disorders n=1	1 (100%)
Endocrine disorders n=5	4 (80%)
Eye disorders n=8	5 (63%)
Gastrointestinal disorders n=3	3 (100%)
General disorders and administration site conditions n=1	1 (100%)
Hepatobiliary disorders n=4	4 (100%)
Immune system disorders n=10	10 (100%)
Infections and infestations n=37	10 (27%)
Injury, poisoning and procedural complications n=2	2 (100%)

Therapeutic Area (MedDRA SOC)	Reviews of Original NMEs that Mention Patient Experience Data
Investigations n=16	0 (0%)
Metabolism and nutrition disorders n=7	6 (86%)
Musculoskeletal and connective tissue disorders n=3	2 (67%)
Neoplasms benign, malignant and unspecified n=79	75 (95%)
Nervous system disorders n=15	15 (100%)
Psychiatric disorders n=9	9 (100%)
Renal and urinary disorders n=9	7 (79%)
Reproductive system and breast disorders n=2	2 (100%)
Respiratory, thoracic and mediastinal disorders n=5	4 (80%)
Skin and subcutaneous tissue disorders n=16	15 (94%)
Surgical and medical procedures n=18	1 (6%)
Vascular disorders n=2	1 (50%)

Appendix B. Glossary

Table 5-2: List of Acronyms

Acronym	Definition
AC	Advisory Committee
BLA	Biologics License Application
CBER	Center for Biologics Evaluation and Research
CDER	Center for Drug Evaluation and Research
CDTL	Cross-Discipline Team Lead
ClinRO	Clinician-Reported Outcomes
COA	Clinical Outcome Assessment
CPIM	Critical Path Innovation Meetings
DDT	Drug Development Tool
FD&C	Food, Drug, & Cosmetic
FDA	U.S. Food and Drug Administration
FDARA	FDA Reauthorization Act
FD&C	Food, Drug, and Cosmetic
IND	Investigational New Drug
INTERACT	INitial Targeted Engagement for Regulatory Advice on CBER/CDER ProducTs
IR	Information Request
NDA	New Drug Application
NME	New Molecular Entity
ObsRO	Observer-Reported Outcomes
PDUFA	Prescription Drug User Fee Act
PerfO	Performance Outcomes
PFDD	Patient-Focused Drug Development
PHS	Public Health Service
PPI	Patient preference information
PRO	Patient-Reported Outcomes
SME	Subject matter expert
SOC	System Organ Class

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