SILENCE IS NOT ALWAYS GOLDEN:
The value of engagement for collection of patient experience data in drug development

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Interactive question #1

What is your work environment?

a) Academia

b) Government

c) Hospital/clinic

d) Industry/consulting

e) Managed care/payer

f) Patient advocacy group

What is patient experience data (PED)?

PED includes the experiences, perspectives, needs and priorities of patients related to:

21st Century Cures Act

- Symptoms of their condition and its natural history
- Experience with treatments
- Patient preferences for outcomes and treatments
- Impact of the conditions on their functioning and quality of life
- Input on which outcomes are important to them
- Relative importance of any issue as defined by patients

FDA

- Impact of the disease and its treatment on patient
- Patients' perspectives about potential / current treatment
- Understanding natural history of the disease or condition
- Views on unmet need

FDA: Food and Drug Administration; PED: patient experience data

1. 21st Century Cures Act. H.R. 34, 114th Congress. Title III, Section 3002(c). 2016. https://www.gpo.gov/fdsys/pkg/BILLS-114hr34enr/pdf/BILLS-114hr34enr.pdf. Accessed April 16, 2024. 2. Food and Drug Administration. Patient-Focused Drug Development: Collecting Comprehensive and Representative Input. 2020. Accessed April 16, 2024.

PED gaining importance for drug development

FDA is continually developing guidance on PED

Pharmaceutical companies have begun hiring chief patient officers

PED focused scientific journals have been published

However, there are still gaps in the overall understanding of PED*

The FDA recently requested information on challenges/limitations from sponsors/academia on PED

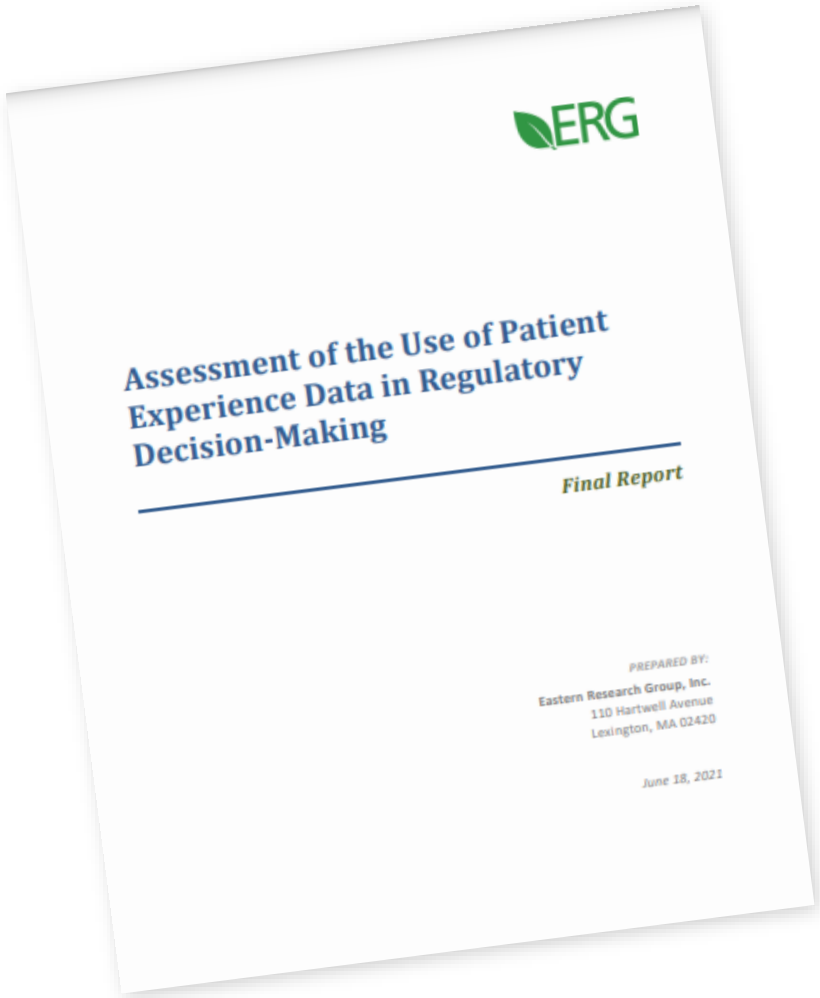
Sponsors want the FDA to provide more direction on:

- PED definitions
- How and when the FDA utilizes PED
- FDA support for the use / importance of PED
- What PED may be incorporated into approved product labeling

*Methodological Challenges Related to Patient Experience Data; Request for Information and Comments

Document ID: FDA-2023-N-1506-0001 https://www.regulations.gov/document/FDA-2023-N-1506-0001

Use of PED in regulatory decision-making



Presentation of patient experience data in FDA reviews*

Form of PED in review documents	% of FDA reviews with PED for Approved NME NDAs and BLAs
Summary of Data FDA presents or describes patient experience data without further interpretation or analysis.	98%
Interpretation of FDA provides comments or conclusions about the relevance of the patient experience data to the review.	48%
Analysis of Data FDA provides its own analysis of patient experience data in a different way than originally presented by the applicant.	15%
Factor in Decision FDA explicitly cites patient experience data in the Benefit-Risk Framework or in other discussions of factors contributing to a regulatory recommendation or decision.	16%

Assessment of the Use of Patient Experience Data in Regulatory Decision-Making, Eastern Research Group, Inc, 2021
https://www.fda.gov/media/150405/download?attachment
*Adapted from Table 2-4. Presentation of patient experience data in FDA reviews

Once upon a time…

This is me holding my first love letter, but I never knew if the letter was opened…

Do you know

why?

Image was generated by AI and refined by AESARA DDX

Let’s talk before it was too late

Table 3-1. Good practices for using patient experience data in regulatory decision-making, as identified by FDA staff, applicants, and other stakeholders (patients, caregivers, clinicians, advocacy/research organizations) interviewed for this assessment

FDA	• Talk with applicants early and often in drug development about good practices in collecting and using patient experience data • Emphasize to applicants the need for a solid analysis plan before gathering patient experience data • Use patient experience data from applicant, FDA meetings, and other sources to provide context, help frame the review, and identify or select relevant and important concepts of interest • Obtain internal COA expertise when needed
Applicants	• Establish a company-wide culture and expectation of patient inclusion in drug development • Talk with FDA early in drug development about good practices in collecting and using patient experience data • Include patients/caregivers in advisory boards to help identify meaningful endpoints and outcome measures, design clinical trials, and review and refine clinical trials in progress • Develop a solid analysis plan for patient experience data, with feedback from FDA
Other Stakeholders	• Collect patient experience data to determine how to make it as easy as possible for patients to participate in clinical trials (to enable generation of data that meets goals for completeness), and to identify what endpoints and other measures are meaningful to patients • Consult with patients throughout entire drug/biologic development lifecycle (to obtain insights that can improve methodologies at every stage of development) • Share results of patient experience data collections with study participants and patient advocacy groups (to maintain positive relationships and obtain additional, ongoing insights)

Assessment of the Use of Patient Experience Data in Regulatory Decision-Making, Eastern Research Group, Inc, 2021
https://www.fda.gov/media/150405/download?attachment

Interactive question #2

In your view, what presents the primary obstacle to early engagement in gathering patient experience data for drug development? (use one word)

What obstacles hinder initiating early engagement?

You may also hear these from stakeholders

Regulator is a naysayer

Regulator is waiting for a full-blown plan

Due to the high unmet needs, we don't need this to convince regulators

Panelist

Selena Daniels, PharmD, PhD

FDA, Deputy Division Director, Division of Clinical Outcome Assessment

Regulatory Perspective

Ebony Dashiell-Aje, PhD

BioMarin, Executive Director & Head, Patient Centered Outcomes Science

FDA-Sponsor Engagement Framework: Patient Experience Data

Natalie Engmann, PhD

Denali, Associate Director, Clinical Outcomes Lead

Practical Experience from a Small Biotech