



PATIENT FOCUSED DRUG DEVELOPMENT

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Patient Experience

Methodologic Guidance Documents



GUIDANCE 1

Collecting comprehensive and representative input

01



GUIDANCE 2

Methods to Identify What is Important to Patients

02



GUIDANCE 3

Selecting, Developing or Modifying Fit-for-Purpose Clinical Outcome Assessments (COAs)

03



GUIDANCE 4

Incorporating Clinical Outcome Assessments into Endpoints for Regulatory Decision Making

04

Methodologic Guidance Documents



GUIDANCE 1

Collecting comprehensive and representative input

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GUIDANCE 2

Methods to Identify What is Important to Patients

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Patient-Focused Drug Development: Collecting Comprehensive and Representative Input

Final Guidance

What is recommended in this guidance?

This guidance provides recommended methods to collect patient experience data that are accurate and representative of the intended patient population.



Why is this guidance important?

This guidance helps to ensure that a systematic approach is used to determine the research design of patient experience studies.



Key Points

- Research methods (including sampling methods) should align with research objectives and questions.
- The research objective defines the target population.
- Study participants should be representative of the target population.



Patient-Focused Drug Development: Methods To Identify What Is Important to Patients

Final Guidance

What is recommended in this guidance?

This guidance provides recommended approaches to identify what is most important to patients' experience with burden of disease/condition and burden of treatment.



Why is this guidance important?

This guidance shows how different research methods can be employed to collect robust, meaningful information on what aspects of symptoms, disease impacts, and other issues are important to patients.



Key Points

- Qualitative, quantitative, or mixed-methods research can be used to identify what is important to patients. Social media may be an approach to collect qualitative and/or quantitative data.
- Questions should be framed in a manner to collect unbiased patient input.
- Patient experience studies should be designed to be inclusive and patient-centered.



Regulatory Use of Patient Experience Data

- Background and context for regulatory reviews
- Benefit-risk assessments
 - Consideration of COAs for trial endpoints
 - Patient Preference Studies





THINGS TO
CONSIDER

General Considerations For Selecting a Research Method



Research
Objective(s) and
Question(s)

Method
Characteristics

Target Population

Expected data

Feasibility of
leveraging existing
literature and data

Time to conduct
study

Study budget

Approaches to Collect Patient Input

	Qualitative Methods	Quantitative Methods	Mixed Methods
Common Research Objectives	Description, understanding and exploration/confirmation	Numerical description, causal explanation and prediction	Multiple objectives; provide complex and fuller explanation and understanding; understand multiple perspectives
Common Study Characteristics	Understand participant views, perspectives and meanings of concepts; study groups and individuals in natural or controlled settings	Study behavior under controlled conditions; isolate the causal effect of single variables	Study multiple contexts, perspectives or conditions; study multiple factors as they operate together
Data Collection	Qualitative data (e.g., in-depth interviews, participant observations, open-ended questions)	Quantitative data generated using structured data collection instruments (e.g., surveys)	Both qualitative and quantitative data