



# **COLLECTING PATIENT EXPERIENCE DATA: HOW YOU CAN BEST HELP FDA?**

VIEWPOINT  
**JAMA**

# Engaging Patients Across the Spectrum of Medical Product Development View From the US Food and Drug Administration



“... the **FDA is working to give patients a greater voice** in medical product development and evaluation.

**Success in these efforts could lead to tremendous advances** in the understanding of health, disease, diagnosis, treatment, and recovery, **ultimately transforming patients' experience of health care** by enabling physicians to tailor care to an individual's specific needs and preferences.

**Including clinical outcomes that are meaningful to patients** can profoundly influence drug development by **ensuring the patient voice is captured.**”

Hunter NL, O'Callaghan KM, Califf RM. JAMA 2015

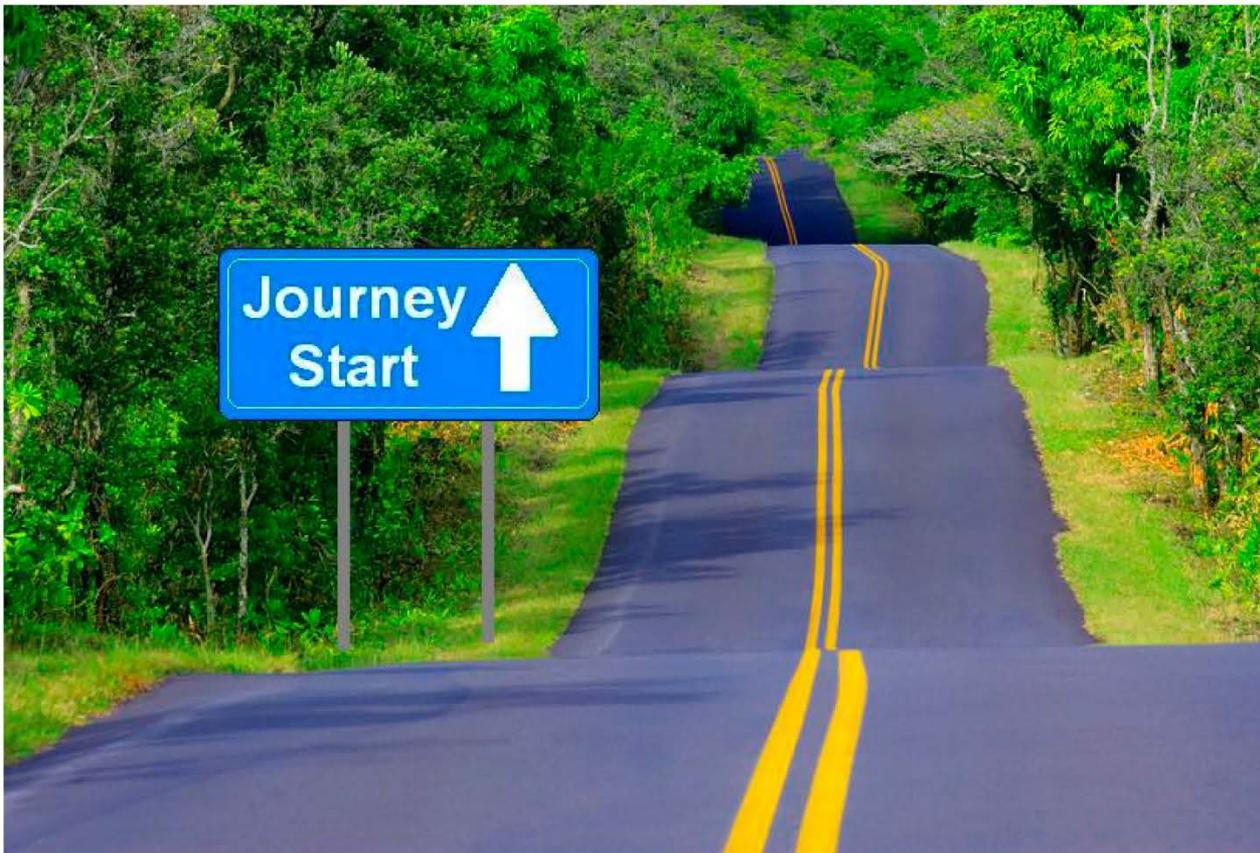
# Our Ultimate Purpose: Understand Patients' Perspectives on Benefits and Risks

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- **Clinical benefit:** A *positive clinically meaningful effect* of an intervention, i.e., a positive effect on how an individual *feels, functions, or survives*
  - How long a patient lives
  - How a patient feels or functions in daily life (includes both improvement as well as prevention/slowing decline)
- **Clinical outcome:** An outcome that describes or reflects how an individual feels, functions or survives
  - Assessed using clinical outcome assessments (COAs)
- Careful assessment of patients' views on benefits and risks are an important part of regulatory decision-making

# Patient Experience

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# What Is Patient Experience Data?

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- Data that are collected by any persons and are intended to provide information about patients' experiences with a disease or condition
- Includes the experiences, perspectives, needs and priorities of patients related to (but not limited to)
  - Symptoms of their condition and its natural history
  - Impact of the conditions on their functioning and quality of life
  - Experience with treatments
  - Input on which outcomes are important to them
  - Patient preferences for outcomes and treatments
  - Relative importance of any issue as defined by patients

Source: Title III, Section 3002(c) of the 21st Century Cures Act

# Where Does Patient Experience Data Come From?

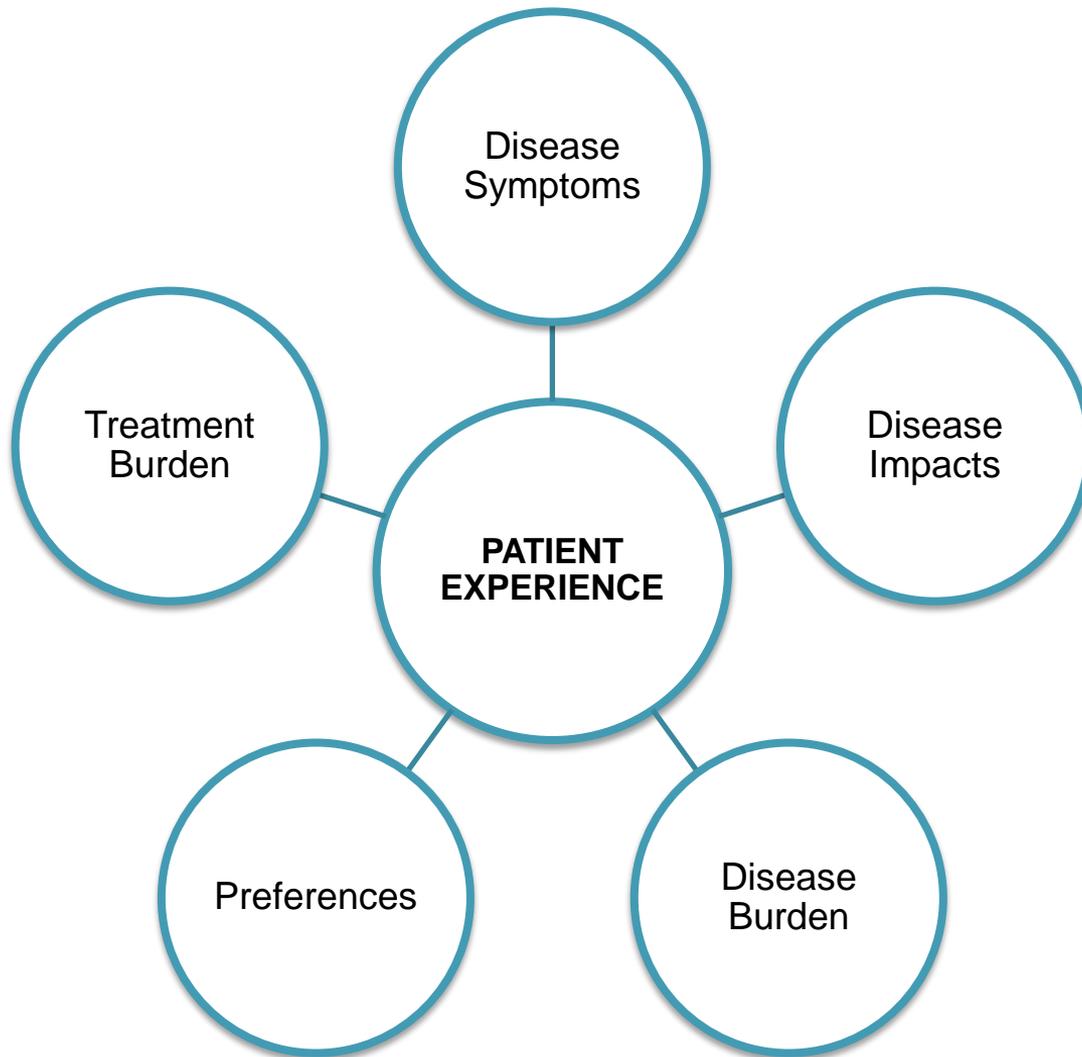
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- The patient's journey should be defined from the patient perspective (where possible) informed by input from patient partners and clinicians

# Patient Partners

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- A **patient** is any individual with or at risk of a specific health condition, whether or not they currently receive any therapy to prevent or treat that condition. Patients are the individuals who directly experience the benefits and harms associated with medical products.
- A **caregiver** is a person who helps a patient with daily activities, health care, or any other activities that the patient is unable to perform himself/herself due to illness or disability. This person may or may not have decision-making authority for the patient and is not the patient's healthcare provider.
- A **patient advocate** is an individual or group of individuals, who may or may not be part of the target patient population, who has a role in promoting an interest or cause to influence policy with respect to patients' health or healthcare.



# How Do You Collect Patient Experience Data?

	Qualitative Methods	Quantitative Methods	Mixed Methods
Method	Uses <b>direct communication</b> to explore or confirm the meaning of interpretation of a topic from the <b>participant's perspective</b>	Uses a <b>tool</b> (survey or questionnaire) that provides <b>numerical information</b>	Uses <b>both qualitative and quantitative data</b> and approaches in an integrated manner in the same study or a set of related studies
Scientific Question*	<i>What aspects of disease are important to patients for measurement and reporting of clinical trial results?</i>	<i>How do we design a questionnaire measuring aspects of disease?</i>	<i>Do we measure symptom severity or frequency?</i>

\*Example questions

Source: Adapted from Teddlie & Tashakkori (2009)

# Why Is It Important To Collect Patient Experience Data?

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- Patients are experts in their own experience of their disease or condition and the ultimate consumers of medical products
- Patient experience data can inform medical product development and enhance regulatory decision making to address patients' needs

# When Do You Collect Patient Experience Data?



- Before and throughout the medical product development process
- Precompetitive collaboration is encouraged!

# Who Can Collect & Submit Patient Experience Data?

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- Anyone can collect and submit patient experience data, including
  - Patients
  - Family members and caregivers of patients
  - Patient advocacy organizations
  - Disease research foundations
  - Researchers
  - Drug manufacturers

# How Can External Stakeholders Submit Patient Experience Data To FDA?

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- Various pathways exist
- FDA guidance on how to submit patient experience data is under development
- Depending on the purpose and type of data, different content and formats may be appropriate



# How Is Patient Experience Data Used For Regulatory Purposes?

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- Patient experience data is used to inform
  - Clinical trial design
  - Trial endpoint development and selection
  - Regulatory reviews including benefit-risk assessments



# Summary

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- Patient engagement is critical throughout the medical product development process.
- You can best help FDA by using scientifically sound methods to collect robust, meaningful, sufficiently representative patient input to inform medical product development and regulatory decision making.



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