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


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The complex tasks of developing, evaluating, and determining the appropriate use of medical technologies occur in an evolving ecosystem of diverse stakeholders. However, as new medical therapies and diagnostics are designed and tested, the preferences and views of the patients and care partners who are most directly affected by these treatments are all too often overlooked. Individual patients often experience different effects of diseases and may have unique preferences about treatments or diagnostic procedures that differ from those of other patients or of their physicians or other health care practitioners. they may also have differing views about what kinds and degrees of risk are tolerable. As patients weigh the balance of benefits and risks, their decisions are informed by their experiences, backgrounds, and personal circumstances. In addition, patients are no longer passive recipients of care; instead, they are empowered consumers of medical products and partners in the process by which those products are developed. Patients increasingly act as advocates for new treatments, and many are fully engaged in making decisions about their care.

Programs recently enacted at the US Food and Drug Administration (FDA) are focused on including patient perspectives throughout the continuum of medical product development.[1] In this Viewpoint, we describe ongoing efforts at several FDA centers and offer views on a conceptual framework within the context of the Precision Medicine Initiative that could lead to improved health by more effectively matching medical products to the needs and preferences of patients and care partners.

New Roles for Patients and Care Partners

The recent emergence of a health care system marked by major structural changes and new technologies and communication channels is offering opportunities for improving medical care. Perhaps the greatest change to accompany this new ecosystem may be the widespread involvement of patients, families, and care partners in every phase of medical product development. Patient advocacy groups[2] are increasingly empowered to participate in this process, and social media offer means for directly engaging patients to elicit input and preferences.

As these developments influence the health care enterprise, the FDA seeks to directly involve patients throughout the lifecycle of medical product development. Although patients today have a greater say in which FDA-regulated products are included in their own care, these new approaches will help to ensure that patient perspectives also have an effect on which medical products are developed and cleared or approved for the market.[3]

Medical Device Programs

The FDA is a member of the Medical Device Innovation Consortium,[4] the nation's first public-private partnership focused on medical device regulatory science. This consortium worked to catalog methods for eliciting patient preferences and to develop a framework that guides research sponsors in approaches for incorporating patient perspectives on the benefits and risks of medical devices for regulatory and other purposes. The FDA, through the Center for Devices and Radiological Health (CDRH) and the Center for Biologics Evaluation and Research, simultaneously posted a draft guidance that provides background information, structure for incorporating patient preferences (defined as qualitative or quantitative assessments of the relative desirability or acceptability of attributes that differ among alternative diagnostic or therapeutic strategies) into regulatory decision making, and advice regarding collection and use of patient preference information for manufacturers and other stakeholders to support certain medical device approvals.[5] This proposed approach stems from the FDA's guidance on benefit-risk determinations for device approvals, which describes patient tolerance for risk and perspective on benefit as an explicit factor the agency may consider in approval decisions.[6] In addition, FDA scientists teamed with behavioral economists to test a method for capturing patient sentiment and to translate it into a decision-aid tool for incorporating patient preferences into clinical trial designs for obesity treatments. Shortly thereafter, the FDA approved a new weight-loss device informed in part by data from this study, the first such device to be approved since 2007.[7]

Partnering Directly With Patients

The recently formed Patient Engagement Advisory Committee (PEAC) is an example of the FDA's commitment to partnering directly with patients. By including patient experts from different backgrounds, the PEAC will help ensure that broad, diverse perspectives on the needs and experiences of patients inform the FDA's deliberations. The PEAC will advise the FDA commissioner on complex issues relating to the regulation and use of medical devices and identify opportunities to partner with patients to meet their needs and support FDA's public health commitment across multiple topics.

Drugs and Biologics

In 2012, the FDA established the Patient-Focused Drug Development (PFDD) initiative to gain a broader range

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"… 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.

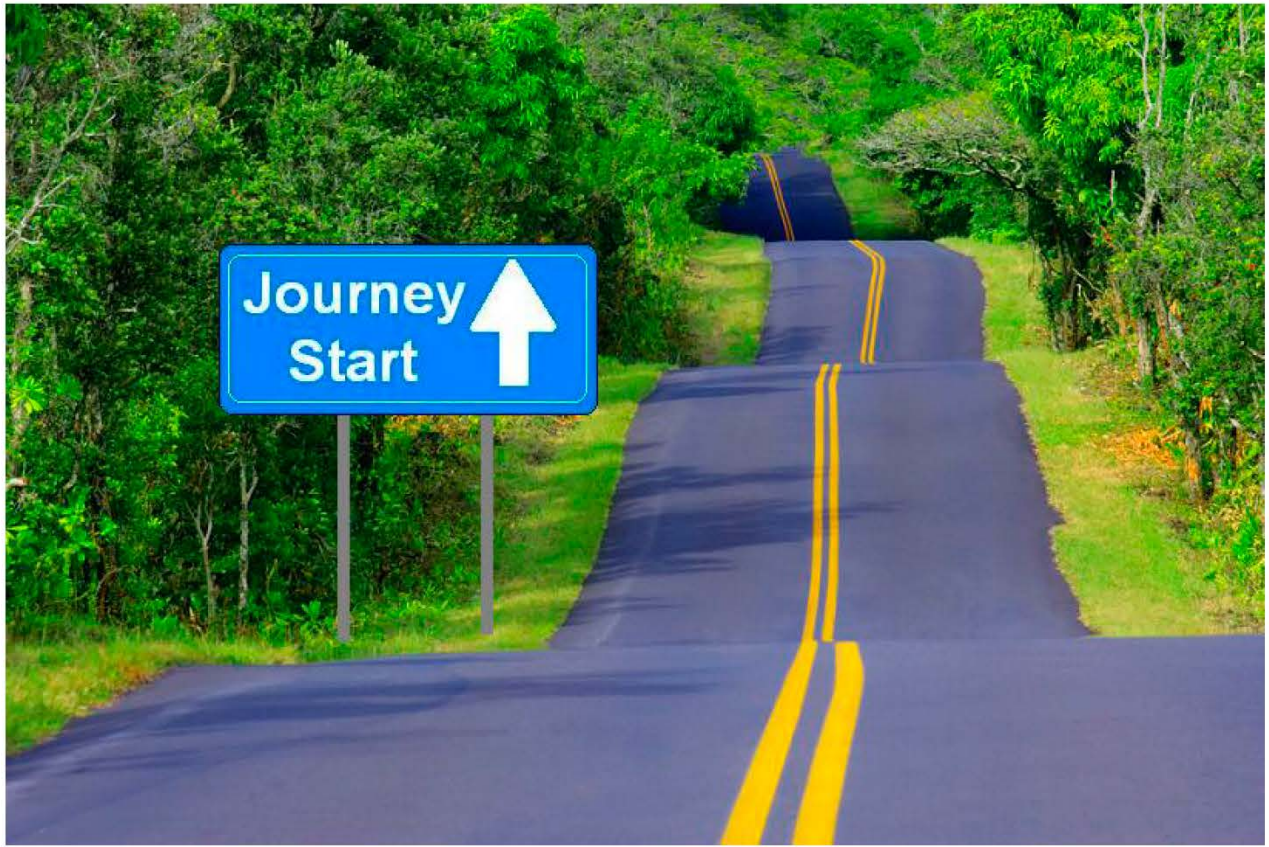
Including clinical outcomes that are meaningful to patients can profoundly influence drug development by ***ensuring the patient voice is captured.***"

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

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

- **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



What Is Patient Experience Data?

- 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?

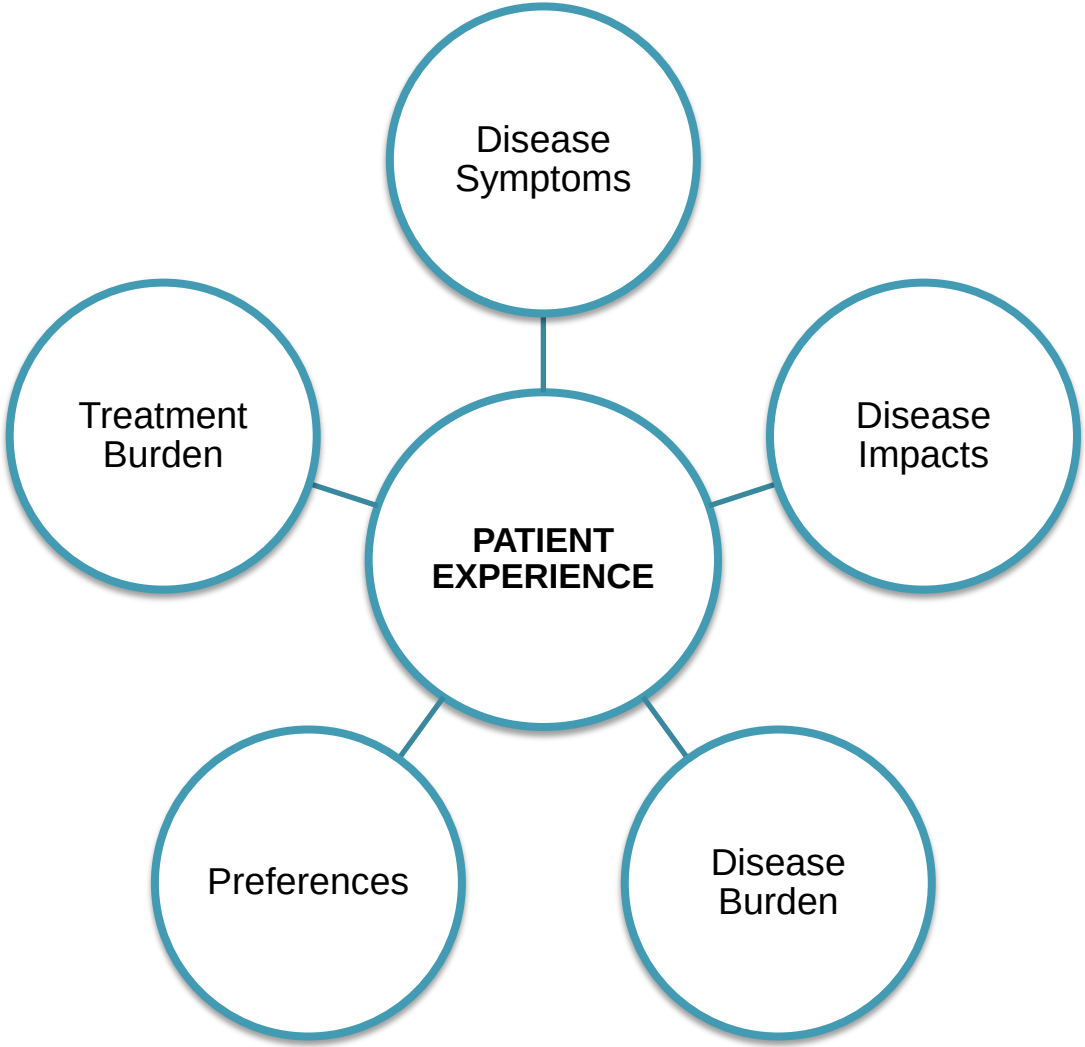
- The patient's journey should be defined from the patient perspective (where possible) informed by input from patient partners and clinicians

Patient Partners

- 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.


Disease Symptoms
Treatment Burden
Disease Impacts
PATIENT EXPERIENCE
Preferences
Disease Burden

How Do You Collect Patient Experience Data?

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

***Example questions**

Source: Adapted from Teddlie & Tashakkori (2009)

Why Is It Important To Collect Patient Experience Data?

- 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?

- 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?

- 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?

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

Summary

- 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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