U.S. FOOD & DRUG ADMINISTRATION

The Regulatory Perspective: **What is Patient Experience Data?**

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THREE OBJECTIVES FOR TODAY

1. Provide examples of patient experience data and its use in medical product development
2. Discuss current efforts to promote and advance the incorporation of patient input into regulatory decision making
3. Identify opportunities for the patient stakeholders to help strengthen capacity and advance fit-for-purpose methods and tools

Introduction to FDA's Medical Product Centers

Drugs	Biologics	Devices
C enter for **D** rug **E** valuation & **R** esearch	**C** enter for **B** iologics **E** valuation & **R** esearch	**C** enter for **D** evices & **R** adiological **H** ealth
Examples: • Prescription • Non-prescription • (Therapeutic biologics) • (Generics)	**Examples:** • Cellular & gene therapy • Tissue & tissue products • Allergenics • Vaccines • Blood & blood products	**Examples:** • Deep brain stimulators • Pace makers & stents • Artificial organs (heart lung & pancreas) • Artificial joints (shoulder, hip, & knee) • MRI, CT scan, lab tests
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Patient Experience Data* (PED)

....data that are:

- **collected by any persons** (including patients, family members and caregivers of patients, patient advocacy organizations, disease research foundations, researchers, and drug manufacturers)
- intended to provide information about **patients' experiences with a disease** or condition, including—

  (A) impact (including physical and psychosocial impacts) of such disease or condition, or a related therapy or clinical investigation; and

  (B)patient preferences with respect to treatment of such disease or condition.

*This definition is from the 21st Century Cures Act: https://www.congress.gov/114/plaws/publ255/PLAW-114publ255.pdf

21st Century Cures Act and PDUFA VI: Topics to be Addressed in Patient-Focused Methodological Guidances

1 **Collecting comprehensive patient community input** on burden of disease and current therapy	➢ How to engage with patients to collect meaningful patient input? ➢ What methodological considerations to address ?
2 **Development of holistic set of impacts** (e.g., burden of disease and burden of treatment) most important to patients	➢ How to develop a set of impacts of the disease and treatment? ➢ How to identify impacts that are most important to patients?
3 **Identifying and developing good measures** for the identified set of impacts that can then be used in clinical trials	➢ How to best measure impacts in a meaningful way? ➢ How to identify measure(s) that matter most to patients?
4 **Incorporating measures (COAs) into endpoints** considered significantly robust for regulatory decision making	➢ Topics including technologies to support collection through analysis of the data

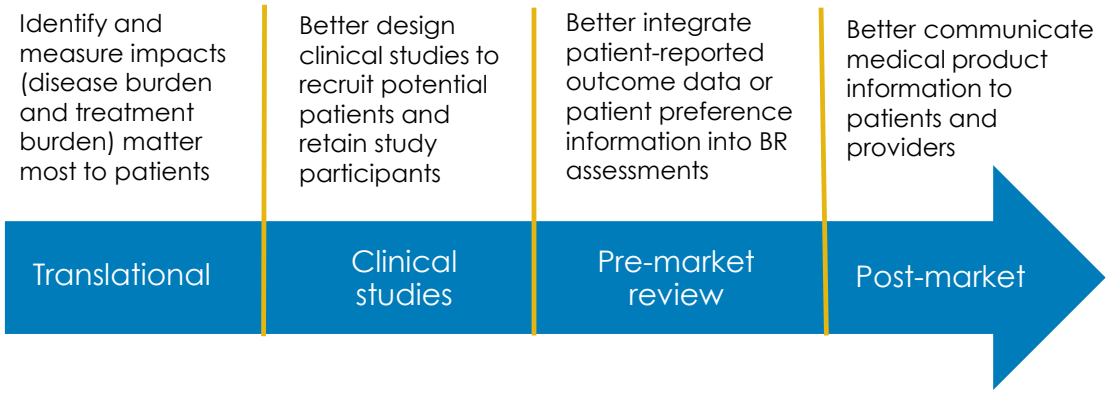
Examples of Questions Related to Patient's Experiences

- What disease impacts matter most to patients?
- How well do the most commonly studied endpoints in clinical trials for a given disease area align with outcomes or aspects of disease that matter most to patients?
- How do attitudes toward or tolerance of potential drug risks or therapy side effects ("preference" considerations) vary by patient subgroup?
- Are currently conducted clinical trials in a given disease area excluding patients who want to be enrolled? If so, why and how might it be addressed?

Examples of Questions Related to Patient's Experiences

- How to modify currently or commonly-used clinical trial protocols to recruit some patients who are otherwise ineligible to participate?
- What measures can be taken to increase the likelihood of patient enrollment in a study and increase the likelihood of participant retention in a study in a given disease area?
- What if any challenges do patients face in trying to adhere to their prescribed drug regimen?
- How well is currently approved labeling communicating the information that patients need to know in order to use drugs safely and most effectively?

Further integrating patient perspective Into medical product development and decision making



Need to build in the patient's perspective starting in the translational phase

How Can Stakeholders Contribute?

- Support/Conduct research
- Natural history development
- Formation of Centers of Excellence in study and treatment of disease
- Policy participation and response
- Coordination
- Communication, Education and Outreach
- Convene meetings and workshops
- Contribution to guidance