FROM ANECDOTAL TO ACTIONABLE:

THE CASE FOR PATIENT PERSPECTIVE DATA

Once viewed as passive recipients of medical products and services, patients are now a vital force for change across the biomedical research system. With patient input in high demand, momentum is building to find more systematic ways of capturing and integrating this transformative resource into decision-making.

To define and scale effective patient engagement requires a paradigm shift, one that is already beginning. Borrowing methods from the fields of health economics, marketing, and engineering, a new science of patient input has emerged, embracing data as a means for measuring patient-centered outcomes and quantifying patient preferences.

The following is a model for advancing the collection and application of Patient Perspective Data, along with initial ideas for how such data might inform drug development and regulatory processes.

Change takes time. There will be progress and setbacks. Those at the forefront of this paradigm shift must work together to shepherd the movement, build the science, and close the gap between the rhetoric of patient-centricity and the reality.

Since its inception in 2003, *FasterCures* has championed patients as partners in the biomedical research enterprise. Our Patients Count program represents more than a decade of work in this space and seizes new opportunities for patients' perspectives to shape the discovery, development, and delivery of medical products. Through Patients Count, *FasterCures*:

- **EXPANDS** the capacity of academics, industry, and patient organizations to build upon the science of patient input;
- **FOSTERS** patient-centric policies and practices that enable greater participation in decision-making; and
- **ADVANCES** the dialogue on the benefits of patient-centricity across the medical product lifecycle.

Look for more tools, templates, and resources in the coming months at **fastercures.org/programs/patients-count**

COLLECTION

DATA SOURCES TO EXPLORE:

DIRECT

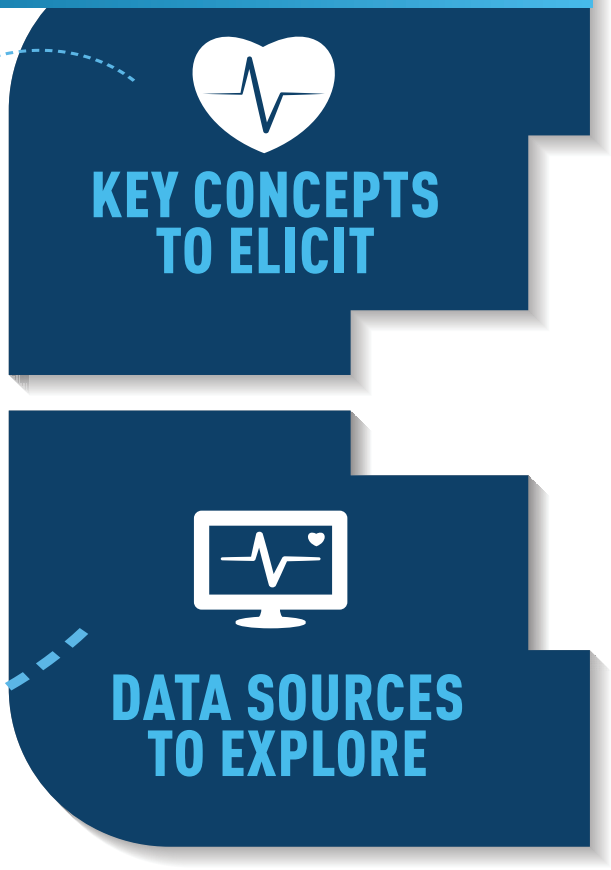
- Unstructured personal narratives
- Focus groups
- Patient advisory boards
- Structured interviews
- Ethnography studies
- Online communities
- Surveys
- Patient registries
- Patient-preference studies
- Clinical trials
- Wearables and mobile devices

INDIRECT

- Social media listening
- Electronic health records
- Adverse event reports
- Claims data
- Published literature
- Conference presentations

KEY CONCEPTS TO ELICIT:

- Symptoms experienced
- Chief complaints (most significant or serious symptoms that cause individual to seek healthcare)
- Burden of managing or living with a condition
- Impacts on daily living and functioning
- Strengths and weaknesses of currently available therapeutic options
- Experience of progression, severity, and chronicity
- Views on unmet medical need
- Minimum expectations of benefits
- Maximum tolerable harms or risks
- Acceptable tradeoffs
- Attitudes toward uncertainty
- Decisions regarding care that patients might encounter



PATIENT PERSPECTIVE DATA CAN BE USED TO:

1) Deepen understanding of the experience of living with a disease or condition, and inform research priorities and resource allocations.

Key applications include enriched understanding of:

- **Disease burden**—the impact of a health problem as measured by financial cost, mortality, morbidity, or other indicators.
- **Patient journey**—a depiction of the typical patient's experience of a condition from early awareness (symptom onset or diagnosis) through resolution or death, which illuminates decisions faced and emotions encountered.
- **Unmet medical need**—the degree to which currently available therapies fail to address the medical needs of individuals, either due to lack of treatments, insufficient efficacy or safety, poorly tolerated side effects, or unacceptable harms or risks.
- **Patient preferences**—tradeoffs that individuals consider or exhibit in making decisions or choices for themselves in decisions about their healthcare. Also refers to absolute and relative importance of attributes, outcomes, or features of a specific medical product.
- **Natural history**—the course a disease takes in individuals from its onset until its eventual resolution through complete recovery or death. Ranks alongside causal understanding in importance for disease prevention and control.
- **Subgroups**—traditionally defined by standard demographic data (age, sex, race, etc.) or genotype, tumor type, prior treatment exposure, etc., patient perspective data can help to identify novel subpopulations grouped by preferences, benefit expectations, risk tolerance, and other experiential factors.
- **Patient-centered outcomes and endpoints**—outcomes from medical care that are important to patients.

2) Influence decisions, plans, and policies that shape the way medical products are brought from microscope to marketplace.

- **Target product profile**—a tool used to plan a drug development program and to facilitate communication between a sponsor and the FDA.
- **Drug development tools**—methods, materials, or measures that aid drug development, including patient-reported outcomes.

APPLICATION

ENRICHED UNDERSTANDING

ENRICHED UNDERSTANDING OF:

- Disease burden
- Patient journey
- Unmet medical need
- Patient preferences
- Natural history
- Subgroups
- Patient-centered outcomes and endpoints

PATIENT-CENTERED ALIGNMENT

PATIENT-CENTERED ALIGNMENT ON:

- Target product profile
- Methods, materials, or measures that aid drug development, including patient-reported outcomes (e.g., measures of health-related quality of life)
- Clinical trial design and operations
- Structured benefit-risk assessment
- Regulatory submissions and decisions
- Patient support programs
- Value strategy
- Formulary selection
- Coverage policy
- Quality measures
- Clinical practice guidelines
- Comparative effectiveness

POLICY OPPORTUNITY

The U.S. Food and Drug Administration (FDA), industry, and patient organizations have identified "expanding patient perspectives in regulatory decision-making" as a top priority in negotiations for the sixth authorization of the Prescription Drug User Fee Act (PDUFA-VI). *FasterCures* has proposed that this definition of Patient Perspective Data guide discussions about the types of information of importance to patients and caregivers.

- **Clinical trial design and operations**—formulation and conduct of studies involving human beings to assess the safety, efficacy, and/or the mechanism of action of an investigational medical product.
- **Structured benefit-risk assessment**—evaluation of the favorable effects or desirable outcomes of a therapy relative to the unfavorable effects or undesirable outcomes conducted throughout the development of a medical product and the basis of FDA's regulatory decision whether or not to approve a product for marketing.
- **Regulatory submissions**—documentation submitted by a sponsor to a regulatory agency for review at various steps of development, including clinical trial applications, fast-track or orphan drug designation, marketing approvals, labeling changes, and annual safety reports.
- **Regulatory decisions**—determinations by FDA at various key milestones along the development pathway for a new medical product and regarding safe and effective use after it is on the market.
- **Patient support programs**—offered by pharmaceutical companies to help patients gain access to approved medications. May incorporate financial assistance and educational support about the condition and care.
- **Value strategy**—demonstration of the personal, societal, economic, and other benefits or gains related to a particular medical product or healthcare service for a given level of resources.
- **Formulary selection**—a payer's or pharmacy benefit manager's preferred list of medications and related products supported by published evidence and expert judgment to encourage the use of safe, effective, and most affordable medications.
- **Coverage policy**—a third-party payer's evidence-based determination of whether a health service (e.g., test, drug, device, or procedure) is proven to be effective or is medically necessary.
- **Quality**—As defined by the Agency for Healthcare Research and Quality, "doing the right thing for the right patient, at the right time, in the right way to achieve the best possible results."
- **Clinical practice guidelines**—recommendations, intended to optimize patient care, which are informed by a systematic review of evidence and an assessment of the benefits and harms of alternative care options.
- **Comparative effectiveness**—evidence on the relative effectiveness, benefits, and harms of different treatment options based on comparisons of available drugs, medical devices, tests, surgeries, or ways to deliver healthcare.

RESOURCES

Learn more about how your organization can take the next step in patient-centricity.

FROM *FASTERCURES*

"Partnering with Patients on Value, Coverage, and Reimbursement" – a summary of our June 11, 2015 workshop

"From Passengers to Co-Pilots: Patient Roles Expand" – an article published in the June 10, 2015 issue of *Science Translational Medicine* that traces the evolution of patient centricity

"Building a Robust Patient Profile of Benefit-Risk in Your Community" – workshop summaries and slides from our Benefit-Risk Boot Camp held September 23, 2014

"Value and Coverage: How Reimbursement Decisions Impact Innovations Needed to Improve Health" – a detailed report and recommendations from our July 8, 2013 workshop

Visit train.fastercures.org for links to a wide array of resources including reports, videos, template agreements, and tools collected from pioneering venture philanthropy organizations.

OTHER REPORTS & WHITE PAPERS

Biotechnology Innovation Organization "A Lifecycle Approach to FDA's Structured Benefit-Risk Assessment Framework" (2015)

Clinical Trials Transformation Initiative "Executive Summary of the PGCT Expert Meeting" (2015) | "CTTI Recommendations: Effective Engagement with Patient Groups Around Clinical Trials" (2015)

Everylife Foundation "Patients As Critical Partners in Rare Disease Drug Development" (2015)

Medical Device Innovation Consortium "Patient-Centered Benefit-Risk Project Report and Catalog of Methods" (2015)

National Health Council "Patient Information Tool & Implementation Guide" (2013) | "Advancing Meaningful Engagement in Research, Development and Review of Drugs" (2015)

Patient-Centered Outcomes Research Institute "PCORI Engagement Rubric" (2014)

KEY FDA GUIDANCES

"Rare Diseases: Common Issues in Drug Development" (August 2015)

"Patient Preference Information – Submission, Review in PMAs, HDE Applications, and De Novo Requests, and Inclusion in Device Labeling" (May 2015)

"Structured Approach to Benefit-Risk Assessment in Drug Regulatory Decision-Making" (February 2013)

"Factors to Consider When Making Benefit-Risk Determinations in Medical Device Premarket Approval and De Novo Classifications" (March 2012)

"Patient-Reported Outcome Measures: Use in Medical Product Development to Support Labeling Claims" (2009)

"Target Product Profile – A Strategic Development Process Tool" (March 2007)

Also see FDA's "Voice of the Patient" report series from the Patient-Focused Drug Development Initiative

COMING IN EARLY 2016

FasterCures is conducting a comprehensive survey of patient-led registries, a vital source of patient perspective data. Look for our report on the current state of registries, as well as recommendations for those considering launching a registry or for making an existing one an even more valuable resource.

FasterCures
A CENTER OF THE MILKEN INSTITUTE

1250 Fourth Street
Santa Monica, CA 90401
Phone: 310-570-4600

1101 New York Avenue, NW, Suite 620
Washington, DC 20005
Phone: 202-336-8900

137 Market Street #10-02
Singapore 048943
Phone: 65-9457-0212

@FasterCures
FasterCures.org