

## PATIENT ENGAGEMENT

# On the path to a science of patient input

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**It is early days in the creation of a science of patient input. Participants are establishing rigorous methods to better integrate patient perspectives, needs, and priorities throughout biomedical and bioengineering R&D and care delivery to patients. To assess progress and unmet needs, *FasterCures* tracked more than 70 collaborative initiatives clustered in six categories that are defining and shaping this developing field. No longer is patient engagement a fanciful notion as it was at the start of our journey in 2003, and the rush of activity is welcome and vital.**

In the 21st century, market research is a business imperative for most industries. In 2011—decades after Steve Jobs famously said, “A lot of times, people don’t know what they want until you show it to them”—Apple started a market research group that sends anonymous surveys to invited users to find out exactly what they want from their devices. In January 2016, IBM formally launched a company-wide process to shift its culture to focus on users’ needs (1). Health care and the research and development (R&D) of biomedical products have lagged behind other technology sectors in moving toward consumer-centered practices. Now, as a result of multiple cultural influences and pragmatic factors, the mindset of these stakeholders is changing, and the patient’s role is expanding (2). Momentum is building to incorporate patient preferences into the biomedical R&D system so that products and services better align with patient needs, improve individual and public health, and reduce time and spending on unproductive care.

With its broad network of stakeholders—patient organizations, industry, academia, government, and funding agencies—*FasterCures* has a distinct vantage point into this landscape of new patient-centered activities; such information is crucial to the creation of a new field: The science of patient input. The goals of this new field are to develop rigorous methods so as to better integrate patient perspectives, needs, and priorities across the translational research continuum. In this Perspective, we summarize and encourage broad use of resources that are already available, and we capture a baseline assessment to benchmark growth and identify areas of unmet need. We don’t want a minute wasted on duplicating efforts.

## WHO’S ON FIRST?

Through an environmental scan, we tracked more than 70 collaborative initiatives, clustered

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**Stakeholders are fashioning of a new field of patient input, which has already begun to take flight.**

in six categories, that are further defining and shaping patient-centered practice and policy (Tables 1 and 2). Within these 70 initiatives, nearly 40 discrete supporting entities are assembling resources, providing direction, and tracking milestones. Each entity approaches this field from a different vantage point, which is what makes the efforts so promising: It is natural—and essential—that the work required to create the field of patient input be performed through strong collaborations composed of highly interactive, diverse organizations.

## FORMING SOLID PLATFORMS: FRAMEWORKS AND MODELS

Some of the first formal efforts to outline the science of patient input borrow, from software development, the use of frameworks to provide a logical structure for organizing information, identifying sources of the information,

and suggesting ways it might be used and viewed by distinct parties (3).

Frameworks serve different purposes, with varied approaches and audiences. It is important to be familiar with these frameworks because they lay the groundwork for much of the ongoing and future work in this space. The Clinical Trials Transformation Initiative (CTTI) created perhaps the most recognizable tool, and its work has become a guidepost. CTTI is a public-private partnership supported by the U.S. Food and Drug Administration (FDA) and member pharmaceutical companies and patient organizations and has popularized a visual chevron-based framework that identifies points at which clinical trial sponsors and regulators might engage patients along the R&D continuum for pharmaceuticals (4). A companion framework for medical devices was developed by another public-private partnership, the Medical Device Innovation Consortium (MDIC), which built detailed considerations into an FDA Center for Devices and Radiological Health (CDRH) diagram of places in the total product life cycle of medical devices at which patient-preference information might enhance product development (5).

The Patient-Centered Outcomes Research Institute (PCORI) requires that all its funded investigators partner with patients from the beginning of the application process through completion of the study and dissemination of its results. To guide formation of meaningful engagements with patients, PCORI developed a Patient Engagement Rubric (6) and a compensation framework (7) that now guide applicants, reviewers, and awardees at every step. The engagement principles outlined in the rubric—reciprocal relationships, colearning, partnership, trust, transparency, and honesty—have become the essential characteristics of patient-centeredness in R&D and health-care delivery. These initiatives, like most of the others identified here, use the U.S. regulatory system as a foundation. Composed of industry and patient groups, the Patient-Focused Medicines Development partnership is leading an effort to develop a comprehensive global framework for patient engagement.

Recently, we have seen a surge in frameworks being used by a number of organizations to help define the value of certain drugs and medical products for insurance coverage decisions. Frameworks assessing the value of medicines have been put forward by the American Society for Clinical Oncology, Institute for Clinical and Economic Review, National Comprehensive Cancer Network, and others; however, most efforts to date have

**Table 1. Resources for the science of patient input.** Shown is a selected sample; please visit: [www.fastercures.org/patients-count-resources](http://www.fastercures.org/patients-count-resources) for a listing of and links to more than 70 resources.

Organization	Frameworks	Methods and toolkits	Regulatory and legislative	Training programs
American Institutes for Research		Roadmap for patient and family engagement health care		
BIO	Lifecycle approach to FDA's structured benefit-risk assessment			BIO annual patient and health advocacy summit
CTTI	Patient engagement across the R&D continuum	Patient group organizational expertise and assets evaluation tool		
U.S. Congress			21st Century Cures Act Patient-focused impact assessment of 2015	
DIA	Visual model of patient engagement in benefit-risk assessment through the medical product life cycle			Patient advocate fellowship program
European Patients' Academy on Therapeutic Innovation				The A to Z of how medicines are developed
Food and Drug Law Institute				Introduction to drug law and regulation
Global Genes		RARE toolkits		
MDIC	Framework for incorporating info on patient preferences	Catalog of methods for assessing patient preferences		
NHC	Advancing meaningful patient engagement (with Genetic Alliance)	Patient info tool		
Reagan Udall				Big data 4 patients

not incorporated substantial input from patients or patient advocates, so we have not included them among the patient-centered efforts in Table 1 and Table 2. To provide more of a patient perspective, *FasterCures* and *Avallere* are partnering to lead a collaborative, multistakeholder process to develop a patient-centered framework for assessing the value of care ([www.fastercures.org/reports/view/56](http://www.fastercures.org/reports/view/56)).

**SOLIDIFYING THE SCIENCE: METHODS AND TOOLKITS**

The science of patient input has roots in multiple disciplines. Patient advocacy builds on

the principles of political activism and community organizing. On the academic side, this new science is attracting individuals trained in health economics, outcomes research, epidemiology, social sciences, and marketing sciences. As a result, there are language barriers among the sectors. There is even disagreement on whether the term “patient” means only the individual with a diagnosis or is meant to include caregivers, advocates, and patient organization representatives. Several initiatives are starting to develop shared definitions, standards, and methods and are collecting tools such as guidelines, principles, checklists, model provisions, and templates.

Over the past 5 years, PCORI has made a substantial investment through its Methodology Committee to create or endorse cross-cutting standards for patient-centered outcomes research for 11 topic areas, including standards for 47 areas such as patient-centeredness, formulating research questions, and preventing or handling missing data (7). Many of these standards can be applied to other fields beyond comparative effectiveness research.

Research on patient preferences is getting attention in part as a result of the draft guidance issued by CDRH in 2015, which spelled out how patient input might inform benefit-risk assessments in premarket applications for medical devices. To help guide industry and other stakeholders on what methods exist for studying patient preferences and when they might be most appropriately used, the MDIC framework includes as a substantial appendix entitled “Catalog of methods for assessing patient preferences for benefits and harms of medical technologies.” This catalog is crucial because many believe that there is only one way to do a patient preference study, and the catalog sorts multiple methods. Similarly, the Biotechnology Innovation Organization (BIO) is working with Parent Project Muscular Dystrophy (PPMD) and a panel of expert reviewers to produce a new resource, “Assessing and integrating patient views into drug development: Patient preference study considerations,” which is due to be released in spring 2016.

Two separate repositories of toolkits aim to help patient organizations standardize some practices and benefit from others’ successes. *FasterCures*’ TRAIN (The Research Acceleration and Innovation Network) program (8) collects resources from leading venture philanthropy organizations, and Global Genes’ Rare Toolkits (9) is designed for use by rare-disease stakeholders. In addition, *FasterCures* held a workshop on 17 February 2016 with key thought leaders from patient organizations, industry, government, and academia in order to identify tools that would enhance the science of patient input, and *FasterCures* will be leading collaborative efforts to prioritize and produce these tools in 2016 and beyond (10).

The National Quality Forum (NQF) announced early this year that it will lead a multi-stakeholder process in 2016 to develop international standards for patient decision aids (11). NQF standards have a potent influence

**Table 2. Science of patient input resources with patient data sources and measurement/metrics.** Shown is a selected sample; please visit [www.fastercures.org/patients-count-resources](http://www.fastercures.org/patients-count-resources) for a listing of and links to more than 70 resources.

Organization	Frameworks	Methods and toolkits	Sources of patient data	Training programs	Measurement and metrics
FasterCures	From anecdote to actionable: The case for patient perspective data	TRAIN Central Station	Expanding the science of patient input: Building smarter patient registries		
FDA		Draft Guidance for Submitting Patient Preference Information	“Voice of the patient” reports	Patient representative program	
Genetic Alliance		PEER platform			
NCATS	CTSA consortium principles of community engagement		Global rare disease registries		
NORD			Natural histories patient registry platform		
PCORI	Patient-engagement rubric	Methodology standards	PCORnet: The national patient-centered clinical research network	PCORI ambassadors program	Patient engagement rubric and evaluation framework
M-CERSI					Assessing meaningful patient engagement in drug development: A definition, framework, and rubric

on the health-care delivery system, and its standards can have far-reaching impact.

**ANECDOTAL TO ACTIONABLE: SOURCES OF PATIENT DATA**

The power of the science of patient input lies in the data, but two key challenges are locating sources of relevant and robust patient data and determining how best to apply them. Data can have limitations and tend to be collected at a single point in time. There has already been enormous investment in standardizing, linking, and mining electronic health records (EHRs), and the promise that EHRs hold for research has been deliberated for years. With U.S. President Barack Obama highlighting the

centrality of data sharing for the Precision Medicine Initiative, and related announcements by major EHR vendors to develop open data standards, progress is being made. But more work is needed to make EHRs reflect the needs of developers, health-care professionals, and patients.

To develop a more holistic picture of the patient journey, new data sources are being identified and leveraged (Table 2). More affordable and prevalent communication and data storage technology has opened up possibilities for patient registries, online data-sharing communities, smart phones, wearable devices, and social media to be used as tools for capturing patient insights longitudinally.

Conventional approaches—such as patient advisory boards, focus groups, surveys, and structured interviews—continue to be mainstays. Capturing dynamic data about domains of high interest to patients will be key to developing better endpoints and outcome measures and understanding benefit expectations, risk tolerance, and attitudes toward uncertainty.

New initiatives are taking shape to fill the need. For instance, in implementing requirements of the 2012 FDA Safety and Innovation Act, FDA’s Center for Drug Evaluation and Research is conducting a series of 24 patient-focused drug development meetings to be completed by the end of 2017. Each meeting is focused on a particular disease or condition and is structured to hear directly from patients and caregivers about the impact of the disease on their daily lives and goals. These sessions also obtain perspectives on how well available therapies meet patients’ needs. For most of the meetings held so far, patient organizations have voluntarily assisted FDA in publicizing the meeting and helping prepare participants by holding, in advance, educational webinars about FDA’s mission and conducting surveys to collect input from a broad patient population. After each meeting, the agency posts a “Voice of the Patient” report (12) as a resource for FDA review teams, industry, patients, and other interested stakeholders. The program’s success led FDA to announce guidelines and an application process for scaling the model with help from other parties to organize and host meetings with the benefit of participation from relevant FDA staff (13).

These FDA-convened meetings have exposed stakeholders to real-world concerns of patients and have allowed FDA reviewers to engage directly with patients about their symptoms and their impact on daily life. This process showcases how patients have complicated and narrowed lives as a result of living with these conditions, and FDA has witnessed tremendous unmet need in a new and powerful way.

Another new source of patient data are patient registries, the explosion of which was documented by *FasterCures* in a February 2016 report, “Expanding the science of patient input: Building smarter patient registries” (14). Adaptable and affordable technology platforms offered by organizations such as Genetic Alliance, the National Organization for Rare Disorders, PatientCrossroads, and Unitio have made it feasible for patient organizations of all sizes to launch registries to study the natural history of disease, burden of disease, expectations

for treatment benefits, and perspectives on tolerable harms and risks. These tools can go a long way to de-risking the science for academia and industry and incenting further study into a particular disease state.

FDA's Office of Surveillance and Epidemiology is leveraging the insights of nearly 400,000 patients who participate on the health data-sharing platform PatientsLikeMe in order to better understand medication side effects and other potential safety issues with approved medications (15). The National Patient-Centered Clinical Research Network (PCORnet), sponsored by PCORI, links 20 patient-powered registry networks with 13 clinical-data research networks at leading academic medical centers, creating a potent infrastructure to compare patient-reported data with data collected in clinical care settings (16). Later in 2016, the U.S. National Institutes of Health (NIH) will announce details for amassing a cohort of 1 million research volunteers through the Precision Medicine Initiative (17). It is anticipated that some of the existing infrastructure supported by PCORnet, NIH's Clinical and Translational Science Awards, and the U.S. Department of Veterans Affairs Million Veteran Program will be tapped to recruit participants, along with direct public outreach in order to achieve a broadly representative cohort.

### **BUILDING A CULTURE OF ENGAGEMENT: REGULATORY AND LEGISLATIVE ACTIVITIES**

In our 2015 article (2), we described a "perfect storm" of policy initiatives that were generating momentum for patient-centered initiatives in government, the private sector, and philanthropies. A year later, these have matured, and new activities are multiplying.

In the U.S. Congress, the 21st Century Cures Act (H.R. 6) sailed through the U.S. House of Representatives with rare bipartisan support, passing by a vote of 344 to 77. The bill contains several provisions that amplify patients' voices throughout the continuum of discovery, development, and delivery of medical solutions. The Senate is advancing a series of smaller bills that address some of the same issues as H.R. 6. The biomedical ecosystem is encouraged by the dialogues from these efforts and looks forward to seeing what will come of them.

In the Executive Branch, the positive experience with FDA's Patient-Focused Drug Development initiative has contributed to the agency's having made a new generation of patient-focused activities among the top

priorities for the sixth authorization of the Prescription Drug User Fee Act (18). Industry, represented by BIO and the Pharmaceutical Research and Manufacturers of America (PhRMA), were reportedly in agreement with the high priority attached to advancing patient-focused drug development, although details about the specific accord reached are still being ratified through the approval processes.

The burgeoning science of patient input has advanced a meaningful culture of patient engagement. For example, CDRH's 2016–2017 Strategic Priorities outline specific milestones such as, "by December 31, 2017, 90 percent of CDRH employees will interact with patients as part of their job duties" (19). This is a strong signal to the medical device industry of CDRH's commitment. Although work remains to define the purpose and means to meet CDRH's target, we believe it will be a transforming force for the agency and also will spur new initiatives in industry. CDRH has also championed patient centrality in its negotiations with industry trade organizations over the fourth authorization of the Medical Device User Fee Agreement, but it is not yet clear how well that priority aligns with industry's top concerns.

Collective success in the science of patient input depends on incorporating new regulatory tools in this field. Unless FDA has capacity both in terms of appropriate methods and expertise to evaluate patient input as well as patient-centered tools and instruments submitted by sponsors, the field might wither. Similarly, industry and patient groups crave more direction from FDA about how it will use the patient input they provide and tools they develop to reflect patients' priorities. Collaboration and communication are vital.

In that vein, a new opportunity for patient organizations to influence regulatory decision-making arose as a result of pioneering work led by PPMD to assemble a large and diverse multistakeholder group to draft regulatory guidance to submit to FDA. The agency used the comprehensive draft as a template, and a year later issued formal guidance to inform drug development for Duchenne muscular dystrophy (20). This success has spurred similar initiatives, including one to stimulate drug development for amyotrophic lateral sclerosis (ALS) led by the ALS Association and another to define a "safe harbor" for engagement between patients and industry and the means by which the patient perspective can be better integrated in drug development (led by the National Health Council and Genetic Alliance).

### **BUILDING CADRES OF EQUIPPED PATIENTS AND PRACTITIONERS: TRAINING PROGRAMS**

As the momentum for the science of patient input builds, we must address the demand for training. Several initiatives have been organized to develop formal curricula and focused professional trainings designed to expand the capacity of stakeholders to participate effectively in advancing the science of patient input.

The most comprehensive of these efforts is the European Patients Academy on Therapeutic Innovation, a consortium of 33 partners funded under the ambitious Innovative Medicines Initiative. Two cycles of an in-depth Patient Expert Training Course conducted over a 14-month period have so far trained about 150 participants (21). An educational toolkit of online materials available in seven languages is intended to reach 12,000 patient advocates across Europe so that they are educated in clinical trial design and health technology assessment.

In the United States, disease-specific programs provide a useful foundation for broader-based efforts sponsored by PCORI through its Ambassador program and FDA's Patient Representative Program. As a follow-up to its late 2015 call to action (22) for more training resources, Friends of Cancer Research is developing a program to facilitate patient involvement in clinical trial design and benefit-risk decisions. The Reagan-Udall Foundation, with funding support from PCORI, has begun a new program, "Big Data 4 Patients," which will create a state-of-the-art patient training program (23). Develop Innovate Advance (DIA) has created a competitive Patient Fellowship program as part of its annual convention to expose patient leaders to topics covered at one of the world's largest gatherings of life science professionals and has designed intensive multiday sessions to foster colearning by life science professionals and patient advocates.

### **MEASURING PROGRESS: METRICS**

To maintain momentum, those dedicating human and financial resources to patient input initiatives feel a pressing need to establish metrics and show a return on investment. A cooperative process led by the University of Maryland Center for Excellence in Regulatory Science and Innovation (M-CERSI) proposes a simple system to assess meaningful engagement in drug development; a rubric that scores engagement is used on several dimensions of activity at different points along the development pipeline (24). CTTI is developing tools

to measure the return on investment of patient engagement in clinical trials, and DIA has partnered with the Tufts Center for the Study of Drug Development to use selected case studies as a basis for quantifying the adoption and impact of patient-centered initiatives. The American Institutes for Research initiated a process early this year to identify measurement principles that are meaningful to patients and their families.

By far, the most advanced measurement system has been developed by PCORI. Its multipart evaluation is designed to assess the overall impact of PCORI, the extent of engagement in research, and the effect of engagement (25). The evaluation system includes visual models, sets of questions, metrics, methods, and sources of data. It incorporates new data collection tools, such as the Ways of Engaging-Engagement Activity Tool (WE-ENACT), in each funded project.

### TODAY'S LANDSCAPE AND BEYOND

On the basis of our research, we have noted some overarching trends and unmet needs.

### Avoid duplication and make your work easy to find

The long-ignored area of patient engagement has quickly become crowded with activity. However, the useful frameworks, models, rubrics, tools, and guidelines that already exist are often hidden from plain view. If you cannot quickly and easily find resources on your own website, it's safe to assume that others outside your organization cannot either. Bringing better visibility to one's completed work and products ready to be used is a low-cost, high-return action step that would help newcomers get started and seasoned practitioners identify where more work is needed. We have created a fully linked version on our website of the resources listed in Tables 1 and 2 to help establish a patient-centricity library.

### We need evidence

Even informal efforts can build an evidence base and document what is working and what is not. It is challenging at this stage to know which efforts are demonstrating positive change and can then be scaled and built into standard operating procedures. We are getting better at incorporating real-world success stories into the narrative of patient centricity, but we need more formal ways to document and share experiences. We must also report failures or speed bumps so that we can all learn from them.

### Help wanted

Although the initiatives we have described in this article are spread among many different organizations and involve scores of partners, a closer look reveals that a small group of expert trailblazers is at the core of many. There is a shortage of academic researchers prepared to hone techniques and methods for turning the patient experience into usable data. We ignore this at our own collective peril. The ranks of well-informed and available patient and advocate experts are also thin compared with the heavy demand for their participation. These shortages place undue demands on the pioneers and could result in burnout, lack of continuity, and, possibly, insularity. We also need to better integrate physicians and other health-care professionals into the dialogue.

### This is not a one-size-fits-all effort; customization is required

Much of the early experimentation with patient-centered action has been rooted in rare disease communities, where there are tightly linked patient networks and highly creative and agile nonprofit organizations. As best practices are established to gather and convert insights into actionable data, it is likely that we'll need to tailor practices to the special features of each affected community, taking into account whether the condition is chronic, acute, or terminal; its prevalence or rarity in the population; the stage of scientific understanding about its cause or pathogenesis; and social dimensions such as how connected or dispersed patients are and whether there might be harmony or discord among the groups that serve the patients' needs.

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