

# PARTNERING WITH PATIENTS ON VALUE, COVERAGE, AND REIMBURSEMENT

A SUMMARY FROM THE JUNE 11, 2015 *FASTERCURES* WORKSHOP



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**Healthcare costs are forcing a re-examination of value at every level.**  
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Challenges facing today's research and development ecosystem require that stakeholders—patients, payers, providers, drug and device developers—break down traditional silos and forge new partnerships. High healthcare costs are forcing a re-examination of value at every level, from the individual to the system as a whole. Despite differences of opinion on how to get there, a common goal across all parties is the ability to benefit from life-changing biomedical innovation at a cost that is sustainable to the system.

On June 11, 2015, *FasterCures* hosted a workshop, “Partnering with Patients on Value, Coverage, and Reimbursement,” which brought together over 100 patients, payers, biopharmaceutical companies, foundations, and providers to facilitate greater understanding of one another's needs and perspectives, and foster more effective partnerships. Part of *FasterCures'* emerging Science of Patient Input initiative—aimed at expanding opportunities for patient perspectives to influence the discovery, development, and delivery of new therapies—the workshop explored themes such as paying for value and looking beyond regulatory evidence.

## OPENING THE LINES OF COMMUNICATION

In the months leading up to the workshop, *FasterCures* conducted a series of discussions with stakeholders across the value and coverage ecosystem to unpack this issue and assess the current climate for collaboration. The stakeholders agreed that they needed to engage on this issue but expressed concern about how to do so effectively. For patient organization leaders, there was also a sense of uncertainty about the payer landscape in general.

Following are five key observations *FasterCures* heard going into the workshop, which shaped the agenda and the discussions and interactions onsite:

**1. Regulatory approval from the Food and Drug Administration (FDA) is no longer a green light to the marketplace.** Manufacturers have to prove value to both patients and payers. Industry must now go beyond safety and efficacy standards and demonstrate real-world cost effectiveness of their therapies.

**2. Patient data could be useful in helping to understand and assess value.** Actionable information can be derived from not only clinical trials, but also patient registries, claims and administrative data, and, in some cases, even specialized mobile health applications.

**3. Payers want predictability.** They want to understand the population for which a medical product is intended, what its uptake is expected to be, and whether it will reduce or eliminate the

need for other elements of care and their related costs. They also need to have the ability to exclude drugs from pharmacy formularies.

**4. There is no clear roadmap for patient groups to engage with payers.** Patient advocacy leaders are often bewildered by the vast and heterogeneous payer landscape. Common questions included uncertainty about when to engage, who to call, and how to present information.

**5. Transparency on all sides is important.** From the payer's view, it's not about drug price; it's about understanding how that price was set. On the other hand, benefit managers should explain their rationale for excluding a product from a formulary, especially to patients who were stable on that product.

## DEMYSTIFYING THE PAYER LANDSCAPE

This workshop presented a unique opportunity for patients to engage with a wide group of payers—including representatives from the Centers for Medicare and Medicaid Services (CMS), Anthem, the Blue Cross Blue Shield Association, and Kaiser Permanente, as well as experts on pharmacy benefit managers (PBMs)—and tackle questions about each other's objectives and processes.

Robert Epstein, formerly the chief medical officer at Medco and now CEO of Epstein Health, kicked things off with an overview of the current payer landscape, outlining in detail the process by which coverage and reimbursement decisions are made, as well

# 1

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# 2

### PATIENT DATA

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# 3

### PAYERS

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# 4

### THERE IS NO CLEAR ROADMAP

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# 5

### TRANSPARENCY

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as the different factors that inform that process. By pulling back the curtains on “the DNA of payers,” Epstein not only set the tone for a transparent conversation, but also created a level playing field for patient groups seeking to influence the process. He offered these insights:

► **Understanding what motivates different types of payers to pay out claims can help patients and patient advocates tailor messaging more effectively.** For example, self-insured employers may be motivated to identify and implement policies that will improve recruitment and retention of employees. And self-insured employers who run businesses where the replacement costs of covering for a sick worker are high may be much more receptive to patient-focused policies that could help keep employees at work more consistently. Managed care organizations, and PBMs, however, might be more motivated by factors that will give them a competitive edge in the market, such as customer satisfaction.

► **Understanding the relationship and interaction between payers and those that administer their benefits is useful.** Pharmacy benefit managers, managed care organizations, and risk-bearing provider entities are among the organizations that may make pharmacy decisions on behalf of primary payers. These organizations vary in their risk-bearing arrangements and what their main objectives are (e.g., reduce pharmacy spending vs. reduce total cost of care).

► **Including patients on Pharmacy and Therapeutics (P&T) committees is one potential way to integrate their voice into formulary recommendations.** As evidentiary standards for formulary decisions increase, patient input becomes more critical. Speakers representing different stakeholder groups nodded to the possibility of including patients on P&T committees. This, however, poses some challenges—such as the validity of one individual representing the breadth of patient experiences and getting one in the first place.

The themes of Epstein’s presentation were reinforced in discussions throughout the day, including the need to find new ways to define and conceptualize value at all levels. Tamara Syrek Jensen, director of



“**smarter spending, healthier people, and better care**”

TAMARA SYREK JENSEN

the coverage and analysis group at CMS, noted that CMS, like many other payers today, is focused on identifying and ensuring value in healthcare, and that evidence is critical to this analysis. Syrek Jensen went on to define value as, “smarter spending, healthier people, and better care.”

## GETTING THE PATIENT’S VIEW

Participants consistently acknowledged the challenge of identifying what is “valuable” to patients. **Although there is a wealth of data around patient experience, with more and more being generated each day, there is no clear mechanism for meaningfully integrating these data into regulatory and policy decision-making.**

Multiple panelists shared how important it was for patients, providers, and caregivers to have honest conversations about the value of a particular treatment or procedure to that particular patient, seeking strong alignment between the patient’s goals for therapy and how a particular approach measures up in terms of the trade-offs between expected benefits and potential risks. Poor match-ups can contribute to low adherence rates or wasted medical resources.

Tanisha Carino, vice president of U.S. public policy at GlaxoSmithKline (GSK), noted that GSK is focused on using existing analytics to improve and enhance engagement with patient groups. GSK is also involved in capacity-building among patient groups so they can participate in discussions about the benefit-risk and value of therapies.

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### INTEGRATING PATIENT INPUT

With targeted resources and a laser focus on the needs of their populations, patient organizations have already greatly enriched basic, translational, and clinical research by academic institutions and industry. Likewise, patient input is now being elicited and integrated more systematically across the R&D spectrum, through vehicles such as the Patient-Centered Outcomes Research Institute, the FDA Patient-Focused Drug Development initiative, and the proposed 21st Century Cures legislation (H.R. 6). Yet there is still no common framework for including patient perspectives across the full continuum, including how coverage, reimbursement, and benefit-design decisions are made by payers post-approval by FDA.

Workshop participants explored the tools, resources, and data that many patient organizations are already generating, and came to a few conclusions about how to advance impactful engagement:

▶ **Patients and their representatives have an important role in providing**

**data to shape product design and utilization.** Patient organizations can serve as honest brokers to educate and provide real-world evidence to regulators and payers about their disease, treatment options (existing and prospective), outcomes, and value to patients. As Tanisha Carino of GlaxoSmithKline put it, “So much of our health happens outside the four walls of the doctor’s office,” and patient-reported data are critical to creating a holistic picture of health and disease.

▶ **Patients and their representatives need to “up their game” to be more effective partners.** Patient organizations of all sizes pointed to the need for more capacity-building resources and a clearer understanding of what types and quality of evidence developers, regulators, and payers are looking for. (This must be accompanied by some new thinking by those groups about the uses and value of patient-reported data, as opposed to the “gold standard” of randomized controlled trials.) In the words of Shami Feinglass of Danaher, “How do we make everyone a savvy patient?”

▶ **There is a need for creative thinking about the best ways for patient groups to engage with payers, following the lead of the clinical development community.** Until very recently, patients had very little input to the regulatory process, with just a single patient appointed to advisory committees that weigh evidence at the very end of the lengthy development process. That is beginning to change

with the creation of the Patient-Focused Drug Development initiative in the FDA’s Center for Drug Evaluation and Research and the Patient Preference Initiative at the Center for Devices and Radiological Health (CDRH). CDRH, in partnership with the Medical Device Innovation Consortium, has catalogued scientific methods of eliciting patient input on the benefits and risks of new devices and has begun incorporating that input into its decision-making. Jeff Shuren, director of CDRH, called this a model for patient engagement by other players in the healthcare ecosystem.

▶ **It is helpful to build relationships early, before there’s an “ask.”**  
Jessica Roth from JDRF described the

### PIONEERING SUCCESSES

We heard powerful examples of the role the Cystic Fibrosis Foundation’s patient registry data have played in driving research and drug development; the National Psoriasis Foundation’s work to standardize clinical outcomes measures for trials and practice in a way that reflected the priorities of patients directly rather than via the providers who care for them; Parent Project Muscular Dystrophy’s development of draft guidance submitted to the FDA for the development and regulatory approval of Duchenne therapies, which was the basis for draft guidance issued by the agency; and JDRF’s funding of a large clinical trial to establish the efficacy of a new device to satisfy payers and help ensure patients would have access to the latest innovation.

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foundation's model of relationship-building before there's an "ask," so payers come to know the organization as a trusted broker of knowledge, and potentially as a partner for helping answer shared questions about new treatments. Ultimately, said Mary Dwight from Cystic Fibrosis Foundation (CFF), "many of the payers' questions are the same questions we want to answer – is this treatment worth the time, risk, and cost?" CFF is working with the Blue Cross Blue Shield (BCBS) Association in an effort to determine how the foundation's patient registry data could be utilized in BCBS health plans' coverage decisions.

## MOVING FORWARD— FIVE KEY TAKEAWAYS

There is still much challenging work to be done to align different stakeholders' perceptions of value, but it is beginning, and there are early successes to encourage more dialogue. All of us, across sectors and diseases, will need to work together to change the status quo. Following are five key takeaways from participants about advancing progress:

### 1. Early engagement with payers can improve alignment between regulatory and post-market evidentiary needs.

Better understanding of payer motivations and evidentiary needs could help industry and patient groups prepare a value proposition. Beyond clinical trial data, peer-reviewed literature, and specialty society guidelines, other forms of real-world evidence and data elements could inform how therapies and care can play out in larger populations.

**2. Both patient organizations and payers need to broaden their views and understanding of each other's role.** Patient organizations are uniquely positioned to tell the whole story about their patient populations, and help assess how each component of patient care fits together. This elevated role for patient organizations requires deeper understanding of trade-offs, both about the benefits and risks of treatment and the costs and outcomes of access. To be credible, their viewpoint must convey an awareness about the tradeoffs for the system as a whole, as well as their particular condition of interest.

**3. Building a common framework for soliciting and using patient input will be critical.** Patient groups are approaching payers in varying ways and achieving varying degrees of success. As one patient leader said, "In 2005, we were with the FDA where we are with payers today. We have a lot of tools in place that need to be adapted." Although payers at the workshop were interested in learning more about patient preferences, experiences, and needs, they lacked systematic processes for soliciting and using that input. Including patients as members on decision-making committees could be a first step.

**4. Data may be the common language.** Many patient organizations have the information and infrastructure to take on a bigger role in demonstrating the unmet medical needs, burden of disease, patient experience, and value of therapies. Whether it's through registry, administra-



“Patients, clinicians, developers, and payers need to get together to agree on outcomes that represent value and what evidence is required to demonstrate it.”

**MARK SKINNER**  
WORLD FEDERATION  
OF HEMOPHILIA

tive claims, survey, or other types of data that may not quite reach the gold standard, patient organizations can contribute to the "tapestry of evidence" that is useful for payer decisions.

**5. It's important that the patient community maintain as united a front as possible, and not break down into disease silos.** The name of the game is collaboration. The responsibility to be more open and cohesive does not just belong to the scientific, regulatory, and payer communities; it's also the duty of patients and their advocates.

For more information and related resources, visit [fastercures.org/vandcreadinglist](http://fastercures.org/vandcreadinglist)

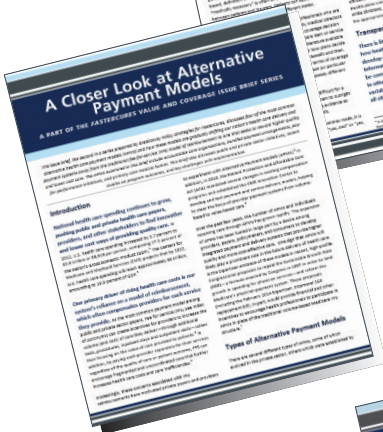
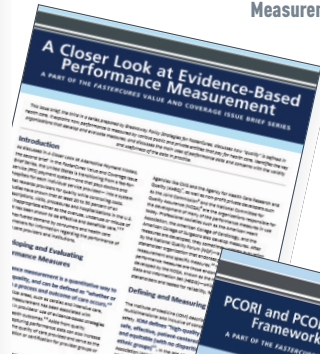
# VALUE & COVERAGE RESOURCE LIST

Following is a list of suggested reading materials on the topic of value and coverage to serve as a companion to the “Partnering with Patients on Value, Coverage, and Reimbursement” workshop summary. These resources explore the role medical research stakeholders, including patient groups, could and should play in providing a framework for an informed discussion about healthcare coverage decisions.

A Closer Look at Health Plan Coverage Policies and Approaches



A Closer Look at Evidence-Based Performance Measurement



A Closer Look at Alternative Payment Models

## Value and Coverage

HOW REIMBURSEMENT DECISIONS IMPACT INNOVATIONS NEEDED TO IMPROVE HEALTH

**Introduction**

The U.S. healthcare system is in a period of dramatic change. As the imperative to control costs has become central, the concept of “value” is discussed everywhere, but its definition and the implications for medical research innovation are unclear. Pressure from payers on biopharmaceutical, device, and diagnostic companies for “real-world evidence”—as opposed to the “gold standard” of randomized controlled trials required for U.S. Food and Drug Administration (FDA) approval—is growing.

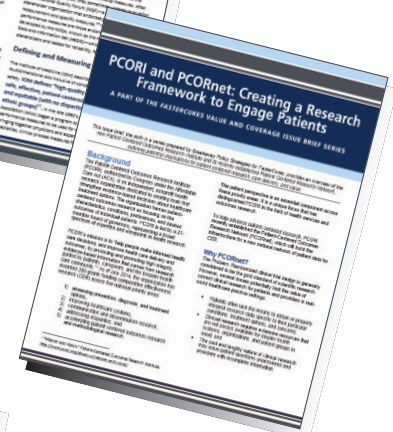
Companies are seeking new ways of showing value to payers and policymakers. Patient organizations are concerned that treatment innovations will be put far out of patient reach—or fail to materialize—if reimbursement issues aren’t tackled.

On July 9, 2013, FasterCures and the Cystic Fibrosis Foundation (CFF) convened a one-day workshop, “Value and Innovation: What Will the New Day Look Like for Patients?” More than 50 leaders of the FasterCures TRAIN (The Research Association and Innovation Network), biotechnology and pharmaceutical companies, payers (public and private), policymakers, and provider organizations met in Washington, D.C. Participants engaged in a candid discussion focused on defining “value” in reimbursement decisions for life-saving therapies. They grappled with issues ranging from the need of payers to methods used to capture patient preferences—all toward the goal of protecting innovation in therapy development. The five themes that emerged throughout the day are reflected in this report:

- Driving value: Patient-relevant outcomes
- Understanding payers’ underlying needs
- Better data-better decisions
- Clearing waste to sustain innovation
- Assessing value: A systems-based approach

At the end, we present recommendations for patient organizations and for other stakeholders engaging with patient organizations to consider.

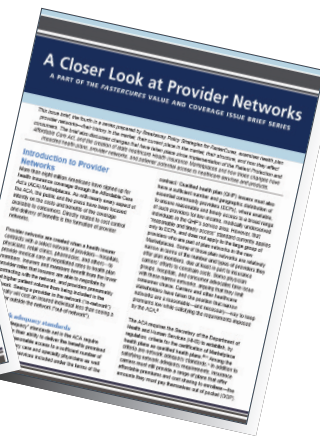
Value and Coverage: How Reimbursement Decisions Impact Innovations Needed to Improve Health



PCORI and PCORnet: Creating a Research Framework to Engage Patients



A Closer Look at Regulatory Developments on Health Plan Coverage and Reimbursement Decisions



A Closer Look at Provider Networks

ADDITIONAL CONTENT AVAILABLE ON THE **FASTERCURES BLOG: [FASTERCURES.TUMBLR.COM](http://FASTERCURES.TUMBLR.COM)**



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