Value and Coverage

HOW REIMBURSEMENT DECISIONS IMPACT INNOVATIONS NEEDED TO IMPROVE HEALTH

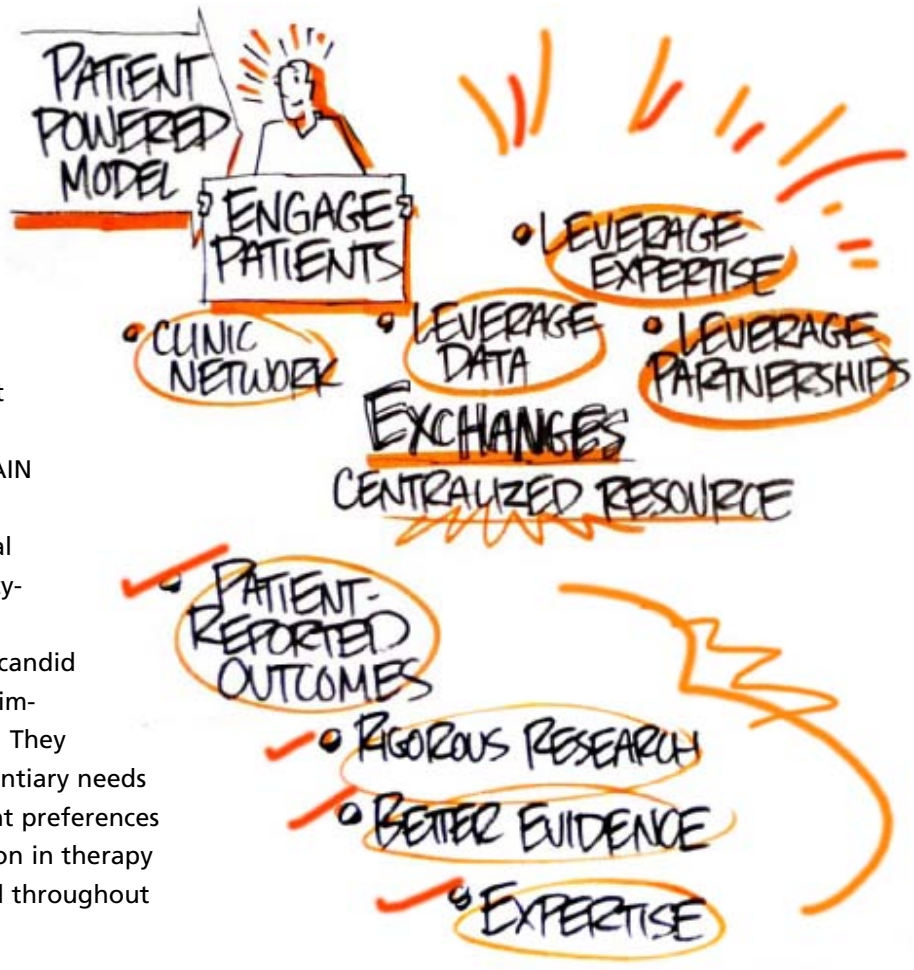
Introduction

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 diagnostics 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 patients' 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 Acceleration 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 lifesaving therapies. They grappled with issues ranging from the evidentiary needs 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:

1. Driving value: Patient-relevant outcomes
2. Understanding payers' evidentiary needs
3. Better data=Better decisions
4. Clearing waste to sustain innovation
5. 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.



Driving Value: Patient-Relevant Outcomes

While it seems obvious that healthcare should reflect the needs of the patients it exists to serve, the concept of patient-centered research and care is actually quite nascent. A 2012 article by Leonard Kish[1] declared "patient engagement" to be the blockbuster drug of the century, a theme that resounded through the pages of the February 2013 issue of *Health Affairs*.[2]

And a February 2013 Institute of Medicine Roundtable on Value and Science-Driven Health Care convened a two-day workshop centered on the central role for patients and families to be leaders in informed care decisions, knowledge generation, and value improvement.[3]

Recent legislation, including the Affordable Care Act (ACA) and the Prescription Drug User Fee Act V, mandates new programs to factor patient experiences into the delivery of

Box 1: Affordable Care Act Basics

The Patient Protection and Affordable Care Act enacted in 2010 aims to increase the quality and affordability of health insurance, lower the uninsured rate by expanding public and private insurance coverage, and reduce the costs of healthcare for individuals and the government. Provisions are being phased in, with many elements taking effect in 2014 and others phasing in by 2016. Here's a recap of the law's major provisions[4]:

- By 2014, almost all U.S. citizens and legal residents are mandated to have qualifying health insurance coverage, or be subject to tax penalty. By 2015, employers with more than 50 employees are required to offer health insurance to employees, or may be subject to a fee.
- Health insurance exchanges opened in October 2013 to provide a marketplace for sale of conforming plans to individuals and small businesses. Credits and subsidies will help individuals, families, and small businesses who meet certain income tests to purchase coverage.
- Medicaid coverage is expanded to low-income individuals.
- Coverage is expanded for pre-existing conditions and young adults who can stay on a parent's plan until age 26. Lifetime limits on essential health benefits are banned for new plans.
- Specified preventive care services are fully covered. Employers are allowed to offer wellness incentives to employees and some may be eligible for grants to create wellness programs. Chain restaurants are required to display nutritional information.
- Bonus payments to primary care physicians and surgeons in underserved areas help address healthcare shortages. Other payment structures emphasize coordinated care.
- New rules for private health insurance policies limit spending for administrative costs. Rate increases, cancellations, and appeals processes are more tightly regulated.
- Supports for comparative effectiveness research, demonstration projects for Medicare and Medicaid "pay for performance" healthcare delivery, and national quality strategies are designed to improve quality and system performance. Funding is also provided for long-term care demonstration projects, healthcare workforce training programs, and community health centers.

As implementation of the 2010 Affordable Care

care and the regulation of drugs and devices (see Box 1: Affordable Care Act Basics). New payment models being pioneered by public and private payers shift the financial incentives from fee-for-service to performance-based compensation in hopes of achieving better patient health outcomes. These changes have led to deeper interest in understanding, measuring, and managing patient health to attain higher quality and lower cost care (see Box 2: Who's Who in the Health Insurance Ecosystem?).

In spite of the prevalent focus on patient needs in current publications and policy, two-thirds of the *FasterCures* workshop participants indicated by survey their belief that patients' needs and priorities are *not* driving decisions made by payers, innovators, and providers.[5] A 2012 survey of 100 pharmaceutical benefits managers conducted by PwC Health Research Institute ranked patients and disease advocacy groups as one of the least important sources of information when making decisions about coverage.[6]

Act proceeds, this question weighs heavily on the minds of policymakers and insurance executives. Beginning in October 2013, health insurance exchanges will give more Americans a direct stake in the question than ever before. Employers large and small will be assessing value as well, looking for new ways to share coverage costs and build a healthier workforce.

Nonprofit organizations that have become engines for accelerating the pace of medical research and advancing new discoveries from the bench to the bedside are recognizing that they have a voice in this value conversation. Patients whose interests they serve are among the medical consumers who must piece together a fractured medical delivery system and pay some or all of the costs associated with diagnosis, treatment, rehabilitation, and long-term care. Nonprofits' constituents rely on them to build relationships with stakeholders from academia and industry, Capitol Hill and governors' offices, single practitioner clinics, and regional healthcare systems so that their interests are represented and protected.

Whether these organizations are focused on fueling research, advocating at the federal or state level, or delivering services directly to patients, they must also look for ways to squeeze time and cost out of the system to improve health outcomes for people living with the conditions they exist to conquer. To do this, they are learning how to approach payers about access and reimbursement issues earlier in the cycle of research and development activities they support. They have built unique positions of trust with their constituencies and have an unmatched ability to engage patients and caregivers in communications and actions that drive change. Such assets are crucial to identifying and implementing solutions that yield systemic and sustainable improvement in the healthcare system.

All the stakeholders in the system agree that innovation is necessary to get to a state of better health for the system and its diverse participants, motivated and pressured by different forces. Innovation may come in the form of new products or better ways of delivering existing ones. It may come from novel incentives or reward systems for creative thinking or compliant behavior. Innovation may result from establishing standards across populations or developing personalized, custom solutions. Fostering innovation in all its forms—throughout all the places from which it might emerge—is a goal shared by patients, providers, policymakers, pharmaceutical companies, and payers alike. It is the locus of consensus that creates opportunity for collaboration and coordinated action.

So, where is the disconnect?

Workshop participants agreed that the gap between the ideal world of clinical trials and the real world of healthcare delivery is wide. Drug development is a tightly controlled process. Once a product is on the market, the same experimental conditions are no longer present. The drug may be accessible to a much broader patient population, and disease progression, aging, comorbidities, and the use of other medications add further complexity across a population. Subjects in a clinical trial may look quite different from patients in a clinic, even if their diagnosis is the same. This can make it challenging for payers to establish efficacy with the same confidence regulators do.

The process for approval of a product is based on safety and efficacy data whereas reimbursement is based on meaningful benefit. The evidentiary standards for therapy development are based on outcomes in a clinical trial for the study population. Payers are hesitant to shed light on what constitutes

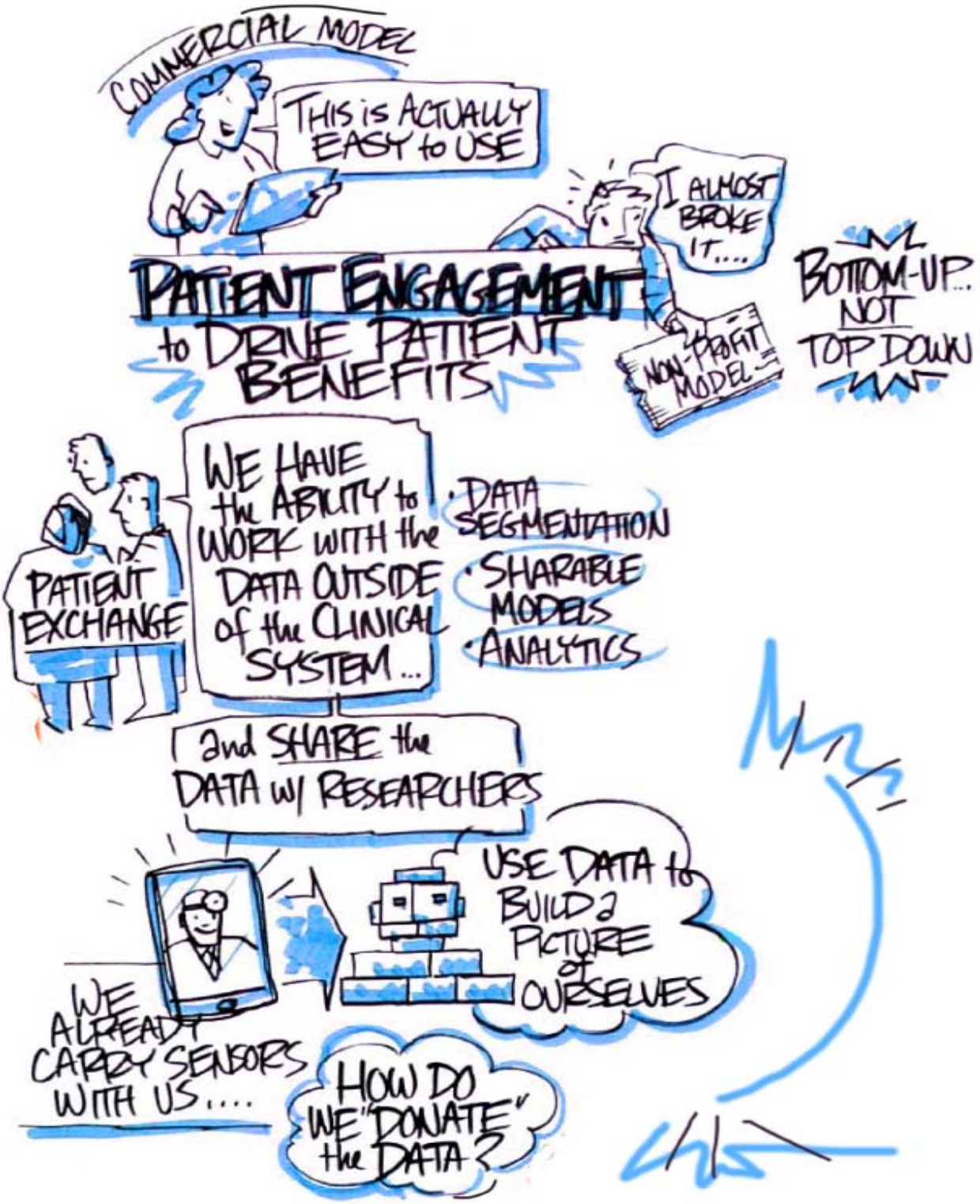
Box 2: Who's Who in the Health Insurance Ecosystem?

While patients are the ultimate recipients of healthcare services, they are not always the customer in transactions involving care that are covered under health insurance programs. This simple fact can create confusion, especially in the reimbursement area. The nation's two largest purchasers of healthcare insurance are the federal government (through Medicare and Medicaid programs) and employers. Medicare and Medicaid each cover about 14 percent of Americans.[7] Fifty-five percent of Americans are covered under insurance plans provided through an employer, and employers pay approximately 75 percent of health insurance premiums.[8]

Some employers administer their own plans, making them the payers as well as purchasers. Determinations for coverage by these employers are internal. However, most employers contract with commercial insurers. In this instance, the employer is the purchaser and the insurer is the payer who administers the plan according to agreed-upon terms of the contract with the employer. Other private insurers, including labor organizations, business coalitions, and consumer organizations, may offer insurance benefits to members, either directly as self-insured entities or by contracting with commercial insurers. With growing numbers of Americans opting for consumer-driven care plans that combine a high-deductible with some form of health savings account, patient-as-payer is taking on new force in the marketplace. Beginning in October 2013, ACA health insurance exchanges will give approximately 12 million Americans the ability to become purchasers. Finally, providers' roles are changing, too. New pay-for-performance models shift risk and some payment decisions to people delivering care.

Healthcare systems are also directly entering the marketplace as payers, offering insurance plans to companies and individuals, and some will participate in health insurance exchanges. As was heard throughout the workshop, the rules of evidence for reimbursement are much more complex today than ever before due to the overlapping—and often confusing—roles and expectations of purchasers, payers, providers, and patients.

meaningful benefit, pushing innovators to expand the ways they measure and report impact on function nd quality of life. Early in the workshop dialogue, a participant offered a view toward a more integrated system of building evidence that starts with health plans telling innovators what outcomes they're expecting to see in a clinical area so researchers can design the trials with that in mind.



Can patients be partners in the process of determining value, as well as consumers? Patients and patient groups have not been seen as hubs of robust datasets. But this is changing. Venture philanthropy organizations have become strategic partners capable of mobilizing large numbers of patients to contribute clinical information, tissue samples, and sought-after "real-world" data about their symptoms, treatment outcomes, and quality of life. They organize clinical care and research networks and take ownership positions in intellectual property and product development. These patient-driven assets are the driving force in some disease areas, as with the Cystic Fibrosis Foundation and T1D Exchange (for type 1 diabetes). These pioneers are also developing evidence to demonstrate value to payers for therapies and care delivery models.

> Establishing safety and efficacy is no longer sufficient when developing a new therapy; innovators must also prove value to patients, providers, and payers in order to generate a revenue stream that covers their R&D costs and provides adequate profits to fuel the next discovery's translation and commercialization.

Technology is equipping patients with new tools to monitor their health. Web platforms allow patients to congregate and voluntarily share and compare symptom notes, treatment insights, and test results from clinical and direct-to-consumer services like personal genome testing. One of the largest forums, PatientsLikeMe, helps users track history and enables members of disease-based communities to generate and test hypotheses and produce outcomes-based data (see Box 3: PatientsLikeMe & the Open Research Exchange).

One workshop participant recounted how cell phones are being used to detect and record hand jitters, disturbed gait, and voice tremor in Parkinson's patients. The data generated predict and segment users quite effectively, creating the potential for "dry biomarkers" emerging directly from patients, completely outside the traditional clinical setting. The Collaborative Chronic Care Network for inflammatory bowel disease at Cincinnati Children's Hospital Medical Center is testing a cell phone app to collect data through the phone's passive sensors and automated user surveys to predict flares so patients might take steps to avert future symptoms.[9]

As one workshop participant put it, "There has been the perception, 'Oh, it's a patient group. Here come the anecdotes.'" However, it is now clear that patients are a rich source of data.

A stark reminder about the responsibility that accompanies greater participation was issued by one participant: "The good news is that the consumer is finally in the driver's seat. The bad news is that the benefits they get will be tied to their choices and their behavior. Everybody in the system now has a need to know what works."

Understanding Payers' Evidentiary Needs

Over the decade between 2001 and 2011 national health expenditures doubled, rising from $1.37 trillion to $2.7 trillion.[10] Today, healthcare costs account for nearly one-fifth of all spending in the United States.[11] With these costs projected to consume an even larger percentage of the federal budget and personal budgets in years to come, payers and consumers are looking for

ways to contain costs without capping quality. This will require that the metrics of quality and value be more transparent and better understood by all the stakeholders in the healthcare system to support collaborative efforts that achieve better outcomes at lower cost.

There is uncertainty about the reimbursement pathway, especially for new products and services as they become available. Establishing safety and efficacy is no longer sufficient when developing a new therapy; innovators must also prove value to patients, providers, and payers in order to generate a revenue stream that covers their R&D costs and provides adequate profits to fuel the next discovery's translation and commercialization. Today it's estimated that a basic science breakthrough takes $1 billion and 15 years to reach patients, if it ever does; only 1 in 10,000 discoveries succeed (see Box 4: Decreasing Venture Capital).[12]

To obtain a favorable national coverage decision for Medicare (the federal government's largest purchaser/payer), innovators must demonstrate that their drug or device is "reasonable and necessary," defined by the Centers for Medicare & Medicaid Services (CMS) as demonstrating that the product or service "improves clinically meaningful health outcomes in the affected Medicare beneficiary population."[13] CMS considers data persuasive when they reflect improvements in the patients' experience of illness rather than changes in laboratory or imaging results, unless they are accompanied by improvements in overall survival, diminished burden of illness, or reduced exposure to painful, invasive interventions.[14] Other payers are not bound by this standard, but CMS's determinations often set precedents for private plans.

> If together we don't build a healthcare system that is affordable and sustainable, we're not going to see the end point of better health that we're all looking for.
>
> **WORKSHOP PARTICIPANT**

At the workshop, the tension between the needs of different stakeholders was evident. Participants

Box 3: PatientsLikeMe & the Open Research Exchange

Inspired by their brother Stephen's journey with amyotrophic lateral sclerosis (ALS), Ben and Jamie Heywood started PatientsLikeMe in 2004 to connect patients with one another so they could share experiences, ideas, and data. In 2013, the online platform crossed the 200,000-participant mark and is now a meeting place for people with more than 1,500 common and rare conditions.

PatientsLikeMe calls itself a "learning health system" and, with support from the Robert Wood Johnson Foundation, is creating the first open-participation research platform for the development of patient-centered health outcomes measures. Access to the platform is free and all instruments and items developed on the platform will be "made openly available for free, unlimited use, and further development with no commercial restrictions."[15] Four pilot research projects selected in August 2013 for design, testing, and sharing on the **Open Research Exchange** platform span palliative care, hypertension, diabetes, and multiple chronic conditions.[16]

Box 4: Decreasing Venture Capital

Uncertainty about how CMS and other payers make coverage decisions is contributing to a significant decline in private investment in the sector, putting biomedical innovation at risk. A 2013 report from the National Venture Capital Association documents the tenuous environment. Venture capital's total investment in life sciences companies has been declining since 2007 with investors moving assets to other sectors where returns are quicker and more predictable. In 2006, 39 life sciences venture funds were raised; in 2012 there were just 11. Competition from other countries that have clarified regulatory and reimbursement pathways and present favorable taxation and intellectual property policies are luring entrepreneurs to shift investments in U.S.-based life sciences innovations to products being developed in other countries.[17]

representing innovators and patient organizations urged payers to be more transparent about the types of evidence used to make formulary and reimbursement decisions. As one said, "Just as you want to reduce regulatory risk by having clarity about what regulatory expectations are, there ought to be ways to reduce reimbursement risk by having more clarity, precision, and consistency about reimbursement evidence expectations."

The market has created a new generation of payers and there are no uniform decision-making standards. Payers described several factors that can constrain the ability to provide clear-cut or consistent answers, such as:

- Self-insured companies, unions, and individuals with high-deductible plans may tailor coverage and payment decisions to reflect local circumstances or unique risk-benefit calculations.
- Health insurance exchanges that opened in October 2013 as part of the ACA are impacting the insurance products that employers and individuals are seeking and what coverage they're willing to pay for.
- Experimentation with accountable care organizations and bundled payments and other forms of value-based purchasing by CMS and private insurers is changing the reimbursement calculus.

Payers underscored the need to keep reimbursement from being continuously additive. "We can't just keep paying for the next new thing without looking to eliminate whatever it is that the new thing replaces," said one payer at the workshop. Reducing wasteful spending and assessing cost across the continuum of care are two topics explored in later sections of this report, as are recommendations for making evidence used to set reimbursement decisions more transparent to all stakeholders.

Better Data = Better Decisions

It is hard to discuss the future of healthcare without a discussion of the role of "big data." At the *FasterCures*/CFF workshop, electronic health records, medical claims databases, clinical trial registries, observational studies, and wearable device tracking reports were just some of the sources of big health data highlighted by participants. Several issues were discussed related to big data.

HIPAA

Protections created by informed consent policies and the Health Insurance Portability and Accountability Act (HIPAA) make some data sets difficult, if not impossible, to access, integrate, and mine. "We're not even sure that it's legally possible to combine and perform computations on some data sets because the various IRBs [institutional review boards] could rule that the data can't be used this way. It's much easier to donate your organs, or blood, or money than it is to donate data," cited one workshop participant.

New HIPAA privacy rules passed in early 2013 allow for "compound authorization" for some types of research but

they also create new barriers for researchers and healthcare systems to perform secondary analyses of protected health information without obtaining consent from individuals.[18] One of the workshop participants raised this as a red flag for innovation: "The new policy is unclear about what it means for for-profit enterprises. If companies cannot leverage data to create services that drive better process improvement, better outcomes, better research, then that is a big barrier to improving quality."

INTEROPERABILITY

Another barrier has been the lack of interoperability of platforms and systems and how they receive data about an individual. As one workshop participant noted, "We don't have even the most fundamental standards that would allow us to snap together data about a patient from the various care providers that they've had over time. We don't have the capacity to connect data coming from their lifestyle devices, their genetic information, and their medical records." Only recently has there been a broad push to develop and adopt data collection standards and institute acceptable data quality thresholds across clinical settings. Federal initiatives such as "Blue Button" and "meaningful use" have provided infrastructure support, financial incentives, and carrot-and-stick approaches to facilitate technology investments and data practices that will yield stronger interoperability (see Box 5: Coming Soon: Meaningful Use and Blue Button).

In academia and industry, there are still strong incentives to build siloed, proprietary data platforms and analytical tools to support research activities. Some federal funding announcements emphasize use of shared technology resources, such as the Research Electronic Data Capture system and the National Database for Autism Research, and there have been other National Institutes of Health-sponsored initiatives to support greater interoperability among institutions and fields. However, a legacy of the investigator-initiated system of grant funding is that billions of dollars have been spent over decades to create single-study data collection and analysis efforts. "How can we design a cheaper infrastructure to do high-quality studies? It's simply unsustainable to spend $300 million for a study with a non-reusable platform," remarked one participant. Data and analyses generated by private companies are fiercely guarded proprietary assets, although there are a growing

> "As payers start to work with more providers on population-based payments instead of fee-for-service, the data and its validity and how rapidly it becomes available will be critical."
>
> **WORKSHOP PARTICIPANT**

Box 5: Coming Soon: Meaningful Use & Blue Button

Adoption of electronic health records (EHRs) promises many benefits to the healthcare system and research, but the U.S. system has been slow to respond. **Meaningful use** is a set of standards defined by CMS that creates incentives for implementing and using EHRs in a meaningful way in the delivery of healthcare. Implementation of meaningful use is staged over five years with its goal to contribute to improved health outcomes by the end of 2016. The **Blue Button** initiative of the Office of the National Coordinator aims to connect patients to their electronic health records, making it easy to download or share medication lists, drug allergies, and test results—at the touch of a button. Blue Button is currently available to veterans, uniformed service members, and Medicare beneficiaries. Other federal agencies and many companies in the private sector, such as UnitedHealthCare and Aetna, are also offering their beneficiaries or members a way to "Blue Button" or download their health data.[23]

number of collaborative data-related endeavors within the pharmaceutical, biotechnology, and insurance sectors (Box 6: Collaborative Approaches to Data Collection). To better understand how this specific model of collaboration operates and its impact on the biomedical research ecosystem, view the *FasterCures*' Consortia-pedia project (www.fastercures.org/consortiapedia).

SHARING DATA

In clinical care settings, there has been limited data sharing across disciplines or across settings (see Box 7: FDA Goes 'Big'). Improvements in healthcare outcomes being reported by collaborative learning networks are attracting vocal advocates, including the Institute of Medicine (IOM),[20] for using data to inform research and deliver higher-quality care (see Box 8: Bridging Gaps). For example, the ImproveCareNow network has increased remission rates for children with inflammatory bowel disease from 48 percent to 75 percent in the first five years, without introducing any new therapies.[21] A workshop participant related this change in thinking and behavior that stemmed from a specialists' participation in a learning health system: "A researcher told me that they used to guard their results so they could publish them. Now every center pools its data, compares outcomes, and learns from one another. He says he'd never go back to the old way again."

Box 6: Collaborative Approaches to Data Collection

The Critical Path Institute, a nonprofit public-private partnership with the FDA, brings more than 1,000 partners from academia, industry, and charitable organizations together to create new drug development tools, including data standards and patient-reported outcomes measures, in precompetitive phases of the translational research pipeline. Another nonprofit member organization, the Pistoia Alliance, facilitates precompetitive collaboration among 63 pharma and life science companies to improve the interoperability of R&D business processes to support innovation. The Health Care Cost Institute (HCCI) conducts independent research and analysis on healthcare utilization and cost data contributed by Aetna, Humana, Kaiser Permanente, and UnitedHealthCare. HCCI aims to understand the factors contributing to rising healthcare costs and to inform policymakers and other stakeholders about ways to derive greater value from the healthcare system.

Box 7: FDA Goes 'BIG'

FDA is currently undertaking several big data projects to integrate and analyze data from product applications, with the objective to provide industry with new information that can be applied to future product development and potentially save billions of dollars in development costs:

- Partnership in Applied Comparative Effectiveness Science (PACES), led by faculty at Johns Hopkins University, facilitates pilot projects to conduct advanced analyses to detect clinical trends to determine which interventions will be most effective for which patients under which specific conditions.
- Janus is an enterprise initiative to improve FDA's management of structured scientific data about regulated products in support of regulatory decision-making.
- Sentinel is a national electronic system that will transform FDA's ability to track the safety of drugs, biologics, medical devices, and ultimately all FDA-regulated products, once they reach the market.[22]

To address the need for better infrastructure and capacity to conduct participatory comparative effectiveness research, in April 2013 the Patient-Centered Outcomes Research Institute (PCORI) issued a series of funding opportunity announcements and other initiatives that will build toward creation of a National Patient-Powered Clinical Research Network (see Box 9: PCORI). One of the unique features of the awards that were made in December 2013 is that grantees must demonstrate preparedness to interoperate with the final awardees, from both governing and technical capacities. PCORI's ultimate goal is for multiple healthcare systems to become more integrated and to obtain richer data on members who receive care inside and outside the participating systems.[23]

Even with new collaborative and interoperable efforts starting to address some of the barriers that exist, few commit to share the product of their labors. The open access movement that has been gathering steam since a 2001 conference in Budapest has been embraced by some 600 institutions. Yet many instruments that would help to standardize collection of data across disease states or populations are themselves restricted for use.

SAMPLE SIZE ISSUES

One final issue raised at the workshop was the slowness of healthcare-related entities to embrace modern informatics and statistical methods employed in other fields like astrophysics and defense. Much of healthcare is based on limited sample sizes and linear statistical analyses of data generated from randomized clinical trials. The computational power available today makes it possible to use non-linear, or Bayesian, statistical approaches to combine objective and subjective data sets. These techniques could also be used to identify patterns in non-traditional data sets such as those generated outside the clinical setting, like online patient forums or mobile health devices. "The same math that segments us for advertising and surveillance purposes is going to work on genetic information and healthcare utilization data; healthcare data isn't the 'snowflake' that some people believe it is," remarked one participant.

Several participants emphasized the chasm that separates the state of healthcare data utilization today and what is needed to drive better decision-making at all steps of the biomedical enterprise. One noted that the day of reckoning is coming fast. A big question that remains unan-

Box 8: Bridging Gaps

Sage Bionetworks grew from roots in one of the first bioinformatics startup companies that used pattern recognition to match genetic variation and function to drug response. Among this Seattle nonprofit research organization's current projects are two open systems to pool data sources and apply contemporary statistical analytical techniques to them, **Bridge** and **Synapse**.

Bridge creates a crowdsourcing marketplace where patient citizens can donate their data, track their use, and engage with researchers to best define and understand the questions important to their community. The initial Bridge pilot projects engage Fanconi anemia and breast cancer communities. A computational challenge on public data about breast cancer generated interest from 150 teams who submitted more than 1 million computational models over six months, with the winning team achieving 77 percent accuracy for predicting relapse.

The Synapse computational platform is a set of shared Web services that facilitates collaboration among scientific teams by providing analytical tools and programming environments where data can be checked in and out with detailed records on operations performed, making it much more than a data repository. It's home to 10,000 data sets, including analysis-ready data sets collected for The Cancer Genome Atlas consortium. Mt. Sinai's School of Medicine will use Synapse for projects on Alzheimer's and diabetes.

swered is whether the types of data being collected in the process of delivering care are clinically relevant and computationally meaningful.

Clearing Waste to Sustain Innovation

With stakes this high, waste removal in healthcare is a big job and a shared responsibility. A 2012 Institute of Medicine report[24] found the $750 billion a year in wasteful spending was the ripest source of funding to fuel innovation. The IOM traced this figure to delivery of unnecessary services, excessive administrative costs, fraud, and other problems. A review of 3,000 treatments by the *British Medical Journal's* Evidence Center[25] found a small proportion of healthcare—just 3 percent—is unequivocally ineffective or harmful. In their assessment, 50 percent is of "unknown effectiveness," meaning there are insufficient randomized clinical trials or other high-quality evidence to support benefits outweighing risks.

Throughout the day the conversation returned to the need to more deeply engage patients and caregivers in building evidence for what works and what doesn't. PCORI's legislative mandate to directly involve patients and other consumer stakeholders in formulating research questions, conducting studies, and disseminating research results is a good start. PCORI may have to overcome strong cultural barriers to medical evidence that appear to limit consumer choice or threaten coverage denials.[26, 27, 28] Reflecting such resistance, lawmakers prioritized comparative effectiveness research in the Affordable Care Act but excluded cost comparison as a parameter for PCORI's research and prohibited its findings from being used as a guide for Medicare and Medicaid coverage.[29]

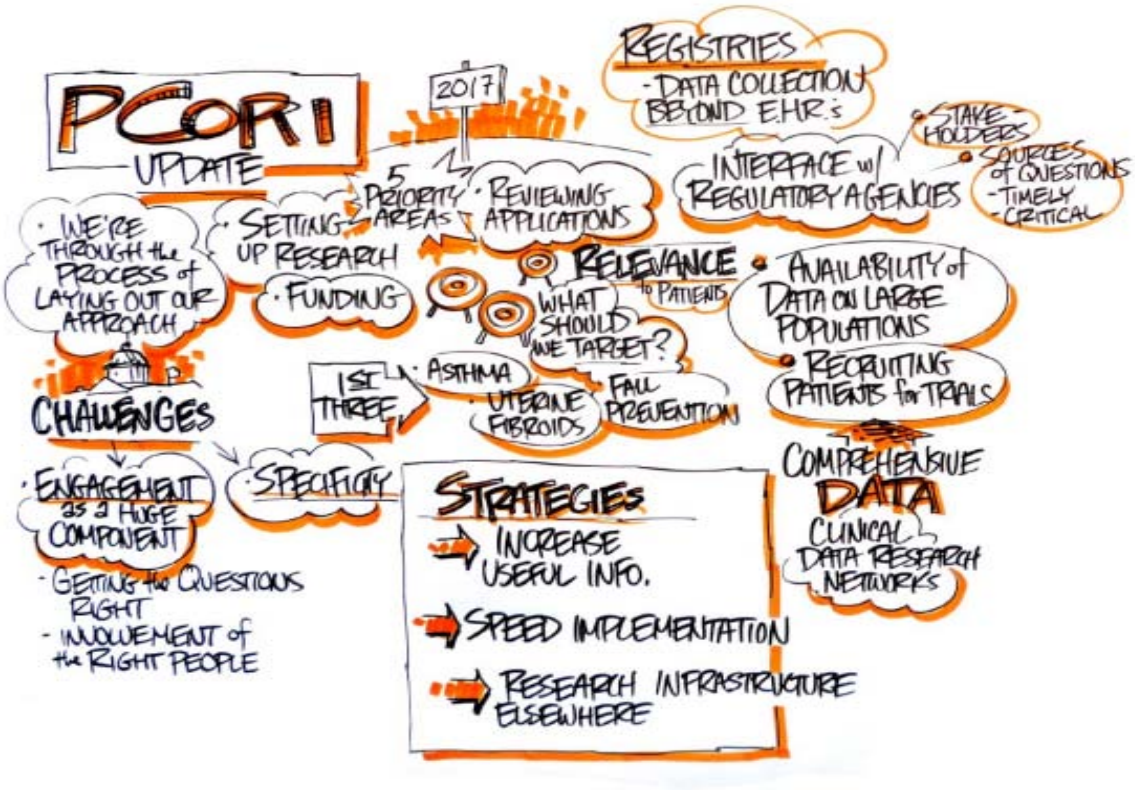
Patient engagement was also viewed as primary to shared decision-making about limited resources and meaningful outcomes. "We desperately need engagement with patients and families to maintain our 'true north' of what matters, to improve the focus on what we measure, and to validate the quality of data we collect," said one payer representative. Another participant put it this way: "If you believe that patients and providers will be on the hook with overall cost, then they will be the ones looking for what's the best way."

Yet, participants acknowledged that fostering patient engagement and informed decision-making on the topic of wasteful spending is challenging, too. A recent IOM roundtable examined the factors that motivate patient action to reduce waste in healthcare and differentiated the incentives of patients with isolated acute care needs from those dealing with chronic conditions. IOM roundtable participants suggested that "understanding the challenges patients [with these chronic conditions] face is fundamental to promoting engagement in healthcare value."[30] Treatment for chronic conditions represents 75 percent of healthcare spending in the United States.[31]

At the *FasterCures*/CFF workshop, it was clear that the venture philanthropy organizations, based on their trusting

Box 9: PCORI

The Patient-Centered Outcomes Research Institute was created in 2010 by the Affordable Care Act to conduct and disseminate research that examines the relative health outcomes, clinical effectiveness, and appropriateness of various treatment and care options. Its $3.5 billion budget through 2019 reflects the U.S. government's heavy investment in research priorities and study design that put patient needs at the center. The funding opportunities issued by PCORI are open to academic, charitable, and commercial organizations alike. PCORI requires that patients and other stakeholders participate in each step of the research process—from proposal development to research design to dissemination of the study results. According to Executive Director Joe Selby, "We engage with patients so we get the questions right and the research right. Involving patients early on will help make sure that results are shared and acted on so that practices change and behaviors change, too."



relationships with patients, are positioned to help broker conversations among stakeholders about effectiveness. "The patient advocacy groups have to be very attentive to how products are used in the marketplace," charged one participant. Some are already functioning in this role. From providing sophisticated disease management services to accrediting care centers to creating decision-making aids for patients and providers, these groups are helping define, disseminate, and encourage best practices in their diseases (see Box 10: Integrating the Patient's Perspective in Determining Value).

Other patient groups are stepping up to the plate or expanding existing programs into areas like treatment adherence that can also yield cost savings and shape better health outcomes. One example is the Cystic Fibrosis Foundation's support of the multicenter observational iCare study that is testing two interventions to improve adherence with prescribed treatments as a means to reduce hospitalizations and improve overall health outcomes.

TRIMMING WASTEFUL SPENDING

Improved adherence sits at the crossroads of patient engagement and a growing trend of value-based insurance design in public and private insurance. Rewarding on-time refills and reducing copays for essential medications and preventative services create incentives for the individual to engage in healthier behaviors, while these up-front expenditures represent an investment in better long-term outcomes. One participant noted, "We're incentivizing consumers to do their part, to be part of the experience and to work collaboratively with plans at the prevention point and with different treatment options that come along the way." Participants also highlighted the need to identify circumstances where innovations can displace less effective therapies in formularies, rather than simply adding to them. "If we're not eliminating things along the way, then we are not really getting at the $750 billion problem. How will we get rid of waste if we can't get rid of what's less effective?"

Greater transparency in pricing products and services was also put forward as having potential to help trim wasteful spending. However, participants raised concerns about the potential for price transparency to have unintended consequences. As one participant noted, "Value must be predicated on quality. When quality information is not available to patients, cost is a surrogate but not always in the direction you'd expect. Sometimes the calculation is, 'If it costs more, it must be better.' So cost and quality have to be part of the same conversation."

> All of you representing the patient community have to be more militant about getting the waste out and not letting waste in. Every wasteful product and service takes dollars away from the exciting things you need done. You must be more militant than anyone.
>
> **WORKSHOP PARTICIPANT**

HEALTHCARE DELIVERY WASTE

Finally, eliminating waste associated with low-quality delivery of healthcare services was identified as an aim for all stakeholders. One participant categorized harm as the extreme low end of the care continuum. Multiple federal and private patient safety initiatives seek to reduce harms and their costly consequences. One illustration, the CMS hospital readmissions reduction program instituted under the ACA, was highlighted as contributing to better attention to patient outcomes and reducing costs. A physician participant described the general harms associated with hospital admissions. "If you're 35 and you're put on bed rest for three days, muscle function declines. If you're 85, it declines even faster and you're put at risk for requiring additional care, additional services, and having reduced quality of life. Every hospital admission puts the patient at risk for harmful events."

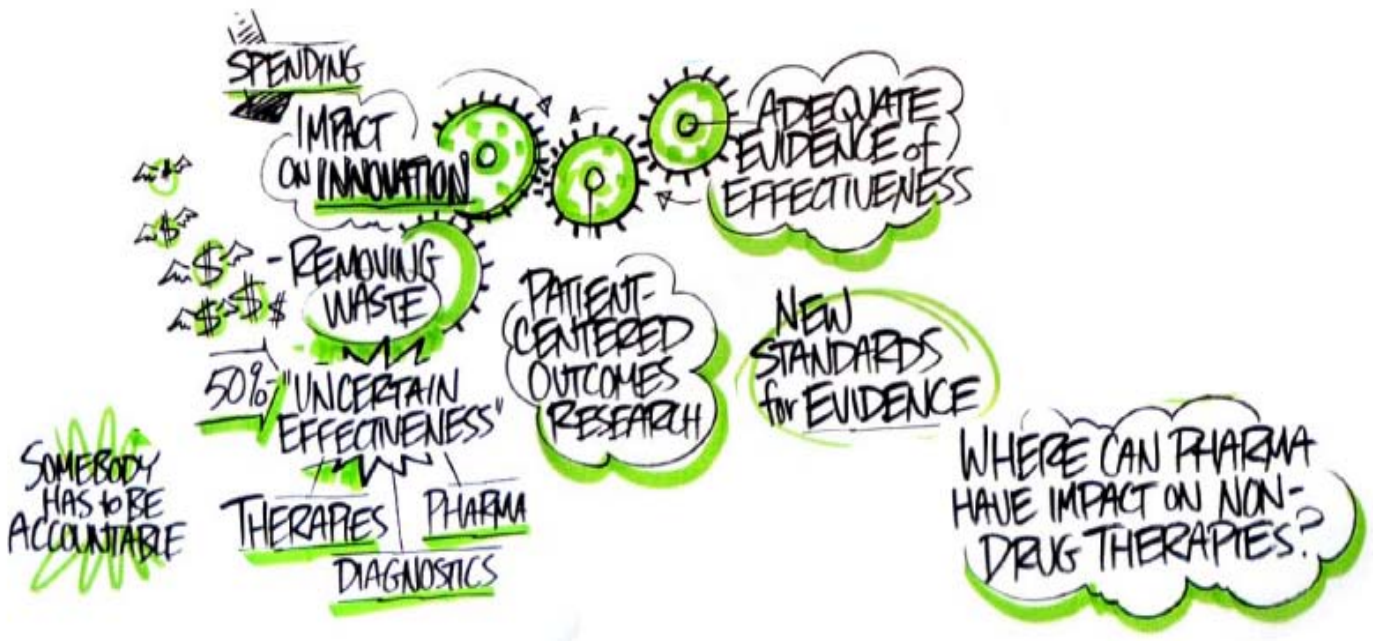
Box 10: Integrating the Patient's Perspective in Determining Value

CYSTIC FIBROSIS FOUNDATION

Since 1955 the Cystic Fibrosis Foundation has fought tirelessly to extend and enhance the lives of those living with cystic fibrosis (CF). Its funding contributed to the 1989 discovery of the defective gene that causes the disease, and it has shepherded development and approval of five drugs that are now part of regular treatment regimens that have tripled the life expectancy of children born with cystic fibrosis. Among its successes is a decade-long partnership with Vertex Pharmaceuticals that resulted in the 2012 FDA approval of Kalydeco, the first treatment of the underlying cause of CF for a subset of patients with a particular gene mutation. The foundation retained a royalty position in the drug that now helps finance development of other compounds for patients who have different gene mutations. Even with all the foundation's accomplishments on behalf of people living with CF, President & CEO Robert Beall spoke of how there is more work ahead. "Patients in our centers do well because they have access to the highest quality of care based on informed treatment guidelines. What keeps me awake is seeing pressures on cost by some states and payers that prevent patients from having access to our centers. We want to make sure the patient has a voice in answering the value question," said Beall at the workshop.

T1D EXCHANGE

For more than a decade Dana Ball directed the investment of more than $200 million in grants for type-1 diabetes (T1D) research for the Helmsley Charitable Trust. "We quickly identified that the number one obstacle was the time and cost of bringing new therapies and drugs to market," he said. To overcome that obstacle, Ball spearheaded creation of the T1D Exchange, an "end-to-end system" that includes a network of 69 clinics with access to more than 100,000 patients, a 26,000-patient registry, a 1,000-patient biobank, and an online social network called Glu, all fueled by partnerships with other charitable organizations, pharmaceutical companies, academic centers,



government agencies, payers, and—most important of all—patients and their loved ones. Now CEO of T1D Exchange, Ball credits patients for the model's power to shave time off every stage of development and delivery. "By having a well-defined network, we can move much more quickly when we have a promising new drug. What concerns me is whether there will be funding to pay for these new therapies with all the pressure on costs I see today."

ALPHA-1 FOUNDATION

John Walsh put patients at the center of the Alpha-1 Foundation from its very start in 1995. He'd been diagnosed with the condition six years earlier and learned that there was no organized effort to promote research or find a cure. Walsh's mother died from the condition and soon after his own diagnosis, his twin brother was diagnosed, too. He stepped in to fill the gap and under his leadership the organization has funded grants in 96 academic institutions around the world and established a patient registry and bank for DNA and tissue samples, a research network, and a successful conference series. He also started a disease management services organization, Alpha-Net, that provides healthcare services to 3,000 patients in 50 states, Puerto Rico, and the Virgin Islands. Alpha-Net's revenues help support the foundation's research program.

AMERICAN HEART ASSOCIATION AND AMERICAN STROKE ASSOCIATION

To combat the nation's fourth-leading cause of death and leading cause of disability, the American Heart Association and American Stroke Association teamed up in 2001 to launch Get With the Guidelines (GWTG)-Stroke, a performance improvement program for hospitals that uses a stroke registry to support its aims. Today more than 1,700 hospitals participate in the program, and data have been collected from more than 2.5 million patient encounters. While its primary goal is to improve the quality of care and outcomes for patients hospitalized with stroke, the GWTG-Stroke program also enables high-caliber stroke research, supports hospital-level quality improvement requirements for CMS, and forms the basis of stroke center certification programs.

The example of hospital-based infections was another source of potential patient harm that could be curbed if all stakeholders were more vigilant about very low-cost interventions like hand washing. "Infection control starts with posting signs in examination rooms to empower patients to ask care providers to wash their hands. Sometimes the bar for participation can be very low."

Discussions about wasteful spending, cost, quality, and value intertwined throughout the workshop. As one participant observed, "When we talk about close to a trillion dollars that we can save from the healthcare system to make room for more innovation, very little of that savings has to do with drug therapies. If we look at this system from the perspective of providing a solution for a patient with a disease, rather than spending on individual products and services, we will have an entirely different view."

Assessing Value: A Systems-Based Approach

There are sources of wasteful spending that can be squeezed out to help balance the equation. But when assessing overall value, workshop participants urged a systems approach to healthcare—looking at the parts in the context of the whole. As one participant stated, "Healthcare today is the whole 'glob'—the diagnostics, the drug, the monitoring technology, the people delivering those things, the setting they do it in, and how they're used by the patient. It's impossible to isolate one thing." Another added, "It's about the person receiving care across multiple episodes, from multiple providers, across multiple care settings."

DETERMINING THE VALUE EQUATION

Workshop participants discussed value to the system

Box 11: Getting REAL with Data

In August 2007, a task force convened by the International Society for Pharmacoeconomics and Outcomes Research (ISPOR) concluded that "real-world data are essential for sound coverage and reimbursement decisions."[32] This declaration reflected the growing need to augment evidence obtained in randomized clinical trials (RCTs) with "studies drawn from the everyday experience of patients who are members of broadly defined populations" and the growing availability of patient-level data. An accompanying editorial framed the need for different types of data: "Unless a [clinical trial] is designed from the start to evaluate coverage and payment, it is unlikely to provide ideal data … to inform broader policy or business decisions."[33]

Clinical outcomes, economic outcomes, and patient-reported outcomes/quality of life were among the types of outcomes needed to augment traditional clinical trials outcomes. The ISPOR task force summarized its findings: "Different study designs can provide useful information in different situations. RCTs remain the gold standard for demonstrating clinical efficacy in restricted trial settings, but other designs—such as observational registries, claims databases, and practical clinical trials—can contribute to the evidence base needed for coverage and payment decisions."[34] Today, a whole industry supports the collection, analysis, and utilization of real-world data. A recent survey of industry professionals conducted by *eyeforpharma* indicates that real-world data is having a medium to high impact on reimbursement, reporting of negative outcomes, developing future clinical trial design, value-based pricing, and—most of all—changing pharma's business model going forward, with 80 percent of survey respondents registering that opinion.[35]

that can be unlocked through greater standardization. Learning health networks that pool data and use continuous improvement processes can help to establish, validate, and/or rapidly refine clinical care guidelines and best practices for patient care and research within a particular condition, across various locations. Alternatively, profiling patients' genotypes and phenotypes according to specific standards can help to more effectively target treatment and preventative services. The pairing of companion diagnostics and specialized therapies to treat certain disease subtypes is advancing in oncology, cardiovascular disease, and other fields. Ideally, both approaches would be employed to maximize the benefit to delivering consistently high-quality, safe, and effective care, adding real-world data to the value equation (see Box 11: Getting REAL with Data).

From a longitudinal perspective, one of the participants emphasized the need to look at value across time from a product or procedure's introduction, to its adoption, to the point at which it becomes routine. Angioplasty was one example given. Another participant pointed out the returns to society that innovation provides even later in the cycle through the generic availability of products, such as with statins, antiretrovirals, and targeted cancer therapies.

Among the intangible factors that warrant a calculation in the value equation is disease prevention, halting progression, and avoiding harms. Costs for services avoided don't appear as a credit to the system, nor do the societal contributions of products and services that preserve or extend life expectancy. One participant observed, "Over the last 30 years, the cost of treating heart disease has tripled in real terms, but the mortality rate has fallen in half. None of us would want a situation where the innovations responsible for saving lives were not adopted by the system, but that's where we're beginning to go today."

Similarly, the economic value of hope as a commodity in care was hard to factor into financial equations, as was the potential for disease cures. As one participant suggested, "If we have two therapies and both do a good job of addressing symptoms of the disease but one also addresses the cause of a disease, doesn't the second one add more value to the individual and the system?" Without the possibility of financial rewards for such ambition, innovators might not take the risk or be able to attract investors to support their research and development costs.

"Part of what's uncertain about much of healthcare is that studies are not conducted with enough focus on patient decision-making or what outcomes clinicians are trying to achieve," said one participant, hopeful that PCORI's studies would help fill that gap.

Another noted, "There's no stack of *Nature* papers high enough to impact patients. If it doesn't benefit patients, that's waste we don't want to introduce." Tradeoffs of defining desired outcomes and value on a condition-specific basis or across broader populations were discussed as well, with some agreement that it might take both—establishing a core set of outcomes and condition-specific measures.

Participants recognized that policy developments as well as technological advances were creating newly aligned incentives and opportunities for collaboration and competition to fuel better value-driven models. Some expressed concern that new pay-for-performance policies might employ a reductionist view of the system, providing compensation based on minimum service levels and lowest-cost therapies, regardless of quality or individual factors. This was of particular concern to those who work closely with complex chronic conditions that lack standardized therapy guidelines or quality measures. "I am concerned about the person who is deteriorating faster than expected and is not responding to the usual therapy. Will there be built-in disincentives to care for that individual?"

High-percentage cost-sharing programs for specialty-tier therapies threatened to put the best treatment options out of reach for patients in some states. "New therapies have thrown our patients a very, very important lifeline and we don't want that lifeline to be frayed by lack of access in the future." It's clear these important considerations warrant new types of interactions and initiatives, especially to effect policy changes that will garner broad support.

Recommendations

FasterCures found that when it comes to the issue of value and coverage for medical innovations, the medical research community is in search of a better understanding of the evidentiary standards that payers consider when reimbursing treatment and care. Top of mind throughout the workshop was this notion that who determines value and what informs that determination are vastly unclear but have large implications for both the cure and care ecosystems.

Greater transparency about evidence for coverage decisions will be vital to helping ensure that we create a reimbursement environment that allows for innovation to thrive and ensures that life-saving therapies are accessible and delivered to patients in a manner that is high quality and cost-effective. As targeted therapies and other innovative medical solutions are entering the pipeline, new healthcare delivery models are starting to emerge, and a panoply of coverage options are under consideration, there is no better time to engage a broad range of stakeholders in this conversation.

At *FasterCures*, we view everything from the lens of the cure system's ultimate stakeholder—the patient. As we sought to identify recommendations that address how access decisions are impacting the innovations needed to improve health outcomes, the following questions guided our thinking:

- How should patient-driven foundations be thinking about their role in driving health improvements for the patients they represent?
- How can they help ensure that innovation is reaching patients and that care is being delivered effectively and efficiently?
- How can they employ their funding, their knowledge of their disease areas and access to patients, and their ability to generate real-world evidence in this new world of value-driven innovation?
- And, how will the other stakeholders benefit from their greater engagement?

Not all recommendations will be appropriate to every patient organization at once. Most depend on the availability of financial, human, and intellectual resources and may be more relevant for organizations at different developmental stages of growth and breadth. Some could be implemented immediately, while others might require longer-term strategic staging and planning. For other parties, these recommendations may be considered as part of due diligence assessments or benchmarking processes.

Similarly, it is critical for *all* stakeholders in this conversation—from payers to providers, from academic scientists to industry executives—to recognize the value of engaging with the patient community and determine effective strategies for incorporating patient perspectives into decisions.

Below are some recommendations for patient organizations and for other stakeholders engaging with patient organizations to consider:

COLLABORATE. Engage with CMS and other payers regularly to increase their understanding of your condition - from the nature of the disease, the impact of existing therapies on quality of life, and even coverage and pricing issues your constituents face. Regular engagement helps inform the decision process and prevent decisions made in a vacuum.

1. Participate actively and openly in conversations with regulators and payers about what value means in each disease/condition. Understand the evidence they are using and/or seeking to make decisions specific to your condition.

2. Establish collaborations between drug/device developers and payers early in the process. At the workshop and in a recent survey by Quintiles, it's evident that payers are seeking involvement in the entire lifecycle of a medicine or device.[36]

3. Participate actively in precompetitive collaborations/ consortia to interoperate with others' systems through selection of data platforms and by developing data standards and shared data dictionaries or other taxonomies.

4. Support open consent platforms to facilitate patients' opportunities to donate data and participate in research.

5. Seek ways to technically interoperate with others' systems through selection of data platforms and by developing shared data dictionaries or other taxonomies. This is especially important for different conditions studied within a particular scientific discipline, such as epilepsy, autism, and multiple sclerosis in the area of neuroscience.

LEVERAGE DATA. Anecdotes are important, but data matter most. Articulate in clear and concise terms what patients consider meaningful to enhance the assessment of clinical benefit.

1. Develop ways to measure and collect data that support patient-relevant outcomes. Develop and/or validate patient-reported outcome measures that can be used to build evidence for regulatory and reimbursement decisions and define/refine clinical care guidelines. Make these available as an open source.

2. Collect data on universal symptom and quality of life domains across care settings in a standardized manner to enable large-scale analysis.

3. Develop and/or enhance patient registries, retrospective data, online communities, natural history studies, etc., to define desired change in quality of life, perhaps at different stages of disease or for different subtypes, in terms that help regulators and payers make informed decisions that reflect patients' values.

4. Explore and understand the risk-benefit continuum in your community using models being piloted by FDA's Patient Preference Initiative[37] and Patient-Focused Drug Development Initiative[38].

DEPLOY BETTER, SMARTER CARE. To ensure coverage for life-saving innovations, you must be vigilant about removing waste from the system and not allowing new forms of waste into it, including misuse of products and services. Challenge behavior that contributes to wasteful spending and lack of accountability.

1. Align incentives by working vigorously to understand what treatments/care approaches work for whom, under what conditions, in which settings, and at what cost, and advocate for the adoption of standardized care guidelines that equalize access to best-practice care for your constituents.

2. Leverage the trusting relationship you have with your constituency to educate about how quality is defined in your condition and the financial realities that innovators and payers are pressured by to facilitate open, informed communication about these challenging issues.

3. Encourage utilization of transparent information sources about healthcare cost and quality per physician, per care setting, and per service.

4. Invest in ways to expand access to, and make better use of, treatments and care that have already been shown to be effective. This includes being informed about the conduct and findings of comparative effectiveness research in your condition.

Throughout this effort, we heard from leaders in the care and cure enterprises that to truly be able to define value, we must integrate patient preferences and expectations throughout the process of scientific discovery and drug development, and healthcare delivery and outcomes measurement.

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FasterCures would like to thank the following people for participating in the workshop "Value and Innovation: What Will the New Day Look Like for Patients?"

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Suggested Reading

The following sources were recommended as background material for the workshop participants.

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