

COMPARATIVE EFFECTIVENESS

Real-World Evidence of Outcomes

Comparative effectiveness research is going to have a huge impact on every stakeholder in healthcare delivery. It requires a much broader view of the healthcare system so that information will exist about a patient's outcome as he or she moves from diagnosis to treatment in the healthcare system.

Comparative effectiveness data are geared broadly to multiple decision makers to provide real-world evidence of the outcomes of various treatments. But challenges remain about developing high-quality data and how those data will be used.

For physicians, comparative effectiveness is going to provide the ability to analyze treatment choices and pick what's best for their individual patients. For patients, it's going to provide them with a lot more information that can help set their expectations for their treatment. For payers, comparative effectiveness will provide support for decision-making about product access and formulary placement that is based on real-world outcomes.

Some experts say this could be a positive for the pharmaceutical industry, providing an opportunity to be seen as creating an improvement to the standard of care. Others worry that comparative effectiveness could be used to determine reimbursement and access. One thing is for sure: pharma companies are going to have to think about the impact of comparative effectiveness and the entire value proposition for their products much earlier in the development stage.

The Research Institute

The reform law establishes a nonprofit corporation known as the Patient-Centered Outcome Research Institute (PCORI). Our experts discuss the implications of this oversight group.

GLIKLICH. **OUTCOME.** The law aims to establish a framework for comparative effectiveness. It designates about \$500 million a year to fund the activities of the PCORI, which is a nonprofit organization that is not technically a government agency. The first order of business is to elect a board of governors, which is what they are currently doing. The second order of business will be to create a methods committee. What's unknown is how PCORI will execute on its mission. There has to be a framework for how all of the different types of studies are catalogued and how the different types of questions that decision-makers have will be answered. We need to have a broader understanding of experimental research, observational research, and modeling and how these relate to each other for various types of questions. If the organization can establish a framework, that would be a starting point so that PCORI and others can look at which projects to fund.

DOYLE. **QUINTILES.** Comparative effectiveness research opens up a new avenue of information gathering around a drug's use and outcomes in the real world. Many types of institutions and companies have launched their own comparative effectiveness studies, some in the public sector and some in the private sector. The law does two things: it catalyzes CER, and at the same time, it recognizes that



A challenge is going to be determining what needs to be evaluated to drive more informed decisions.

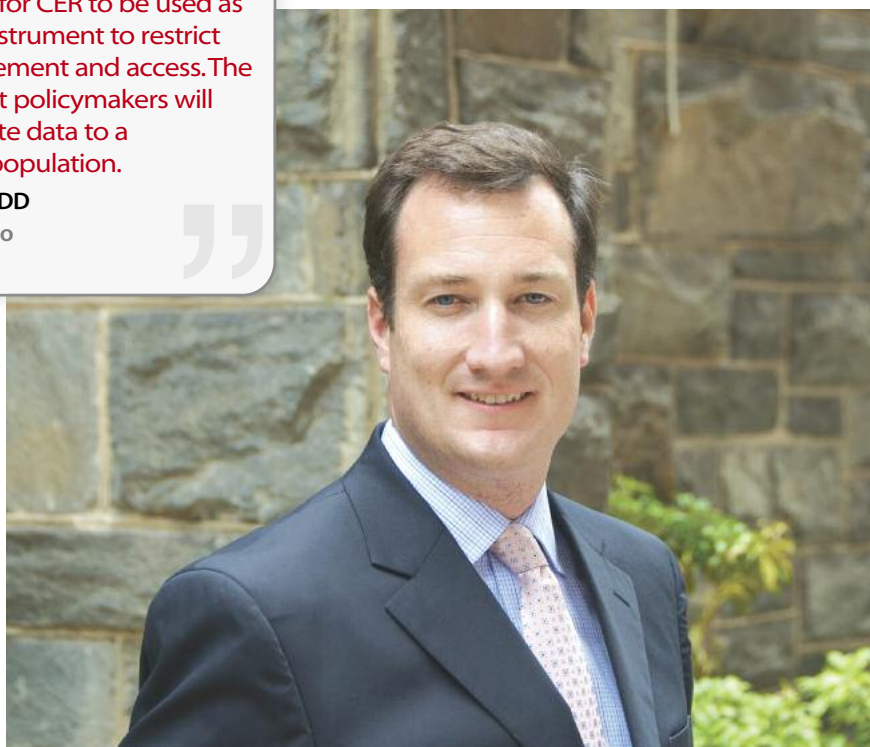
DR. KEN KRAMER
Alpha & Omega



The Patient Protection and Affordable Care Act, passed in March, builds on last year's stimulus bill, which had already **allocated \$1.1 billion to investigate comparative effectiveness** or a look at patient outcomes in real-world settings. The healthcare reform law provides additional funding and sets up a nonprofit institute to determine priorities and provide direction.

The biggest issue is the potential for CER to be used as a blunt instrument to restrict reimbursement and access. The fear is that policymakers will extrapolate data to a broader population.

DANIEL TODD
EMD Serono



there have to be some rules to the road. PCORI is a quasi-governmental institute with some representation from the marketplace that will prioritize the research agenda. What is needed is a methodical and systematic way to prioritize the different initiatives. The institute needs to be open-minded and look to outside experts from the private sector for ongoing research. I would suggest that the organization also look outside the United States to try to learn from other agencies that are evaluating the real-world performance of technologies. This should be viewed as natural progression of a product's investigation. One of the reasons this new institute is important is that payers do comparative effectiveness research; and some of that research they share publicly and some they don't. The CMS also is using this type of information to make coverage and reimbursement decisions, such as 'coverage with evidence development' policy decisions. There should be some convergence between the economics, CER, and evaluating the value of technology compared with the parallel track of the FDA's evaluation of safety. We need to

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Comparative effectiveness research will affect the gamut of drug development, starting with who is enrolled in the trial and how the data are collected.

DR. BILL CROWN
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do a better job at evaluating real-world drug performance in terms of risk-benefit and comparative effectiveness.

KECKLEY. DELOITTE. The passage of this legislation is the convergence of three major trends. First, there has been an explosion of science. There are about 80 randomized controlled trials today being published somewhere in the world. Second, we have more clinical analytic tools now. There are entire departments devoted to clinical bioinformatics, and researchers are mining clinical data warehouses to determine probabilities to develop various types of algorithms and predictive models. Third, and I think most significantly, we have now an impetus in the health reform movement toward the use of electronic health records, and that provides a means of capturing real-time data. In addition, there is the overriding question of cost. This is where the PCORI comes in. The question is: does what we do to treat patients

FAST FACT

Despite spending more on care than any other industrialized nation — \$2.4 trillion in 2008 — the United States lags behind other countries on many measures of health, such as infant mortality and chronic disease burden.

INSTITUTE OF MEDICINE

matter or does what we do just drive up costs. The institute is a straightforward and appropriate effort to mine all of the science that is being created with all these tools and make the data publicly available. PCORI and the Agency for Healthcare Research and Quality (AHRQ) will sit side by side. AHRQ will continue with its traditional model of working with academic institutions to look at the science and conduct studies. This new 21-member group will look at comparative effectiveness from the viewpoint of various industries.

ATKINS. MERCK. The quality of decision-making around treatment and coverage and reimbursement — whether on an individual or societal basis — will be improved if there are more and better data comparing the therapies that are available for any particular condition. Drug companies receive approval for their products by establishing efficacy in trials in controlled settings. These randomized, controlled trials are necessary for establishing causal relationships and isolating the effect of a single intervention, but they are not sufficient to understand how that treatment may work in the general population or to know the circumstances under which one treatment may be preferable to another. There is a lack of high-quality evidence on comparative effectiveness at this point — particularly evidence comparing different types of interventions, such as surgery and drugs, for example. Often payers are in a position of having to decide on covering a widely adopted procedure or intervention and they really don't know whether it improves health outcomes. Payers make judgments now on innovations with a limited amount of information. Everyone in the health system will be better served with more and higher quality evidence. The new, independent coordinating body, PCORI, is well-designed to pull together resources and to get support from stakeholders across the system. It is an important step forward, but it is only one piece of a large enterprise already in place. PCORI's contribution will, hopefully, be to develop research standards, focus resources where the evidence



It would be unnatural and unlikely for Medicare and commercial payers not to try to find a way to use comparative effectiveness information to guide coverage.

LEE BLANSETT
Kantar Health

need is greatest, and raise the level of research activity and quality.

TODD. EMD SERONO. A main objective for PCORI will be to determine the best means to disseminate information to patients and providers and work with many stakeholders to establish priorities. This is a narrower mandate than some earlier proposals. There is great promise in comparative effectiveness, but there is also great threat. There is the challenge of establishing broad policy, which has the potential of limiting access to treatments. As we all know, typically treatments aren't a one-size-fits-all therapeutic solution. There doesn't seem to be an appetite in this country to go toward a NICE (National Institute for Health and Clinical Excellence) model used in the United Kingdom, where cost-effectiveness analysis is used to restrict patient access and limit reimbursement. There was \$1.1 billion in the 2009 stimulus funding for comparative effectiveness, and AHRQ got \$400 million of these funds. Those projects will continue and funding will be fully allocated by September 2010. AHRQ's role will likely increasingly be focused on the dissemination of information.

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Comparative Effectiveness

The Department of Health and Human Services defines comparative effectiveness as the conduct and synthesis of research comparing the benefits and harms of different interventions and strategies to prevent, diagnose, treat, and monitor health conditions in real-world settings.

The purpose of this research is to improve health outcomes by developing and disseminating evidence-based information to patients, clinicians, and other decision-makers, responding to their expressed needs, about which interventions are most effective for which patients under specific circumstances.

A report released last year from the Institute of Medicine (IOM) recommends 100 health topics that should get priority attention and funding and spells out actions needed to ensure that a comparative effectiveness research initiative will be a sustained effort with a continuous process for updating priorities as needed and that the results are put into clinical practice.

But there is a disconnect between the central tenets of evidence-based healthcare and the knowledge, values, and beliefs held by many consumers, according to a recent study by Health Affairs.

Editor's note: Please see the digital edition of PharmaVOICE for more on these studies.

The one thing that AHRQ lacked was the authority or the input from the private sector to set research priorities. PCORI is the national body that will set priorities. Again, the danger of CER is over-simplification. In terms of our healthcare system, we have to keep this in context. Drug costs are only about 15% of overall healthcare costs. Certainly CER holds great promise for advancing knowledge about medical intervention and is going to be a good tool for understanding and getting better value for healthcare dollars, but it is not going to be the silver bullet to solve our healthcare cost problems.

CARO. UNITED BIOSOURCE. The new institute could be either a terrific force or it could be a waste of money and time. This is going to depend a lot on how PCORI is led. The United States has an opportunity to innovate and not take the same pathways that other countries have taken.



A concern that I have is what criteria are going to be used to determine comparative effectiveness.

NICK COLUCCI
Publicis Healthcare
Communications

Pharma and Comparative Effectiveness

Our experts discuss the impact of comparative effectiveness on the industry, from development through marketing.

TODD. EMD SERONO. Companies are going to have to think about the impact of comparative effectiveness and the entire value proposition for their products much earlier in the development stage. With or without federally funded comparative effectiveness, this is the direction the industry is going. Certainly, there is going to have to be more effort to differentiate products on label.

ATKINS. MERCK. More widespread use of comparative effectiveness research in healthcare decision making can stimulate more attention to comparative risk and benefit in development. Placing the contribution of the new drug therapy in the context of treatments that are already available and understanding how that contribution varies for subgroups of the population will have an impact on companies' go/no-go decisions on development. Companies will gain a competitive edge by processing that information earlier and making decisions on the basis of better information.

DOYLE. QUINTILES. Comparative effectiveness provides pharma companies with an enormous opportunity to be rewarded for their innovation but it also gives them a responsibility to continue to update the profiles of their products. A drug's approval is just the beginning. Companies have to start to create new channels of information gathering in the



The question is: does what we do to treat patients matter or does what we do just drive up costs?

PAUL KECKLEY
Deloitte

real world. This has already started with mandated REMS and the FDA safety Sentinel Initiatives. But the industry should not have to wait for a mandate for comparative effectiveness; companies need to come up with new techniques to follow their products and know how the products are being used, where they fit in the healthcare system, and how they're performing over time. They also need to create a continuous feedback loop to their development programs to design a better product going forward.

KRAMER. ALPHA & OMEGA. Comparative effectiveness is going to produce better treatment because more information is going to be available up front. If a brand is going to be evaluated based on comparative effectiveness research, it only makes sense for pharma companies to do comparative effectiveness proactively instead of having third parties do retrospective analyses. Comparative effectiveness analyses will ultimately help pharmaceutical companies develop the strongest argument for informing physicians why their treatment is the best option for patients. For marketing companies, instead of just producing a detail aid that talks about x percentage increase or decrease of something, it will perhaps extend the discussion to include additional outcomes that are going to have expanded meaning. The latter will certainly speak to the sensibilities of those with interests in managed care.

CARO. UNITED BIOSOURCE. If the comparative effectiveness movement heads in the right direction, it will be extremely beneficial because it could help guide the development of products to meet healthcare needs. The bad thing that can happen is for comparative effectiveness to develop the way it has in the United Kingdom. The system there sets up perverse incentives that companies then respond to, whatever they are. If they are told that to be covered, a product has to meet a cost-effectiveness ratio of x then companies do everything they can to meet cost-effectiveness x. They are not likely to develop products that don't meet that standard because it will be impossible to get them covered. A better approach — and hopefully the United States will move in this direction — is to evaluate what the needs are in the population and set the system up so that companies feel compelled to try to fulfill those needs because they are rewarded better if they do.

GLIKLICH. OUTCOME. This is the first year we've seen receptivity and understanding among a broad group of stakeholders that if



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DR. JOHN DOYLE
Quintiles Consulting

Good CER Practices

There is a growing recognition that randomized clinical trials alone will not fill the information gaps caused by the escalated demand for data to support decision making. Experimental studies, such as clinical trials, provide the strongest level of evidence because the groups being compared are similar except for their randomization to treatment. Observational studies are more challenging to analyze and interpret because the various reasons that doctors and patients choose different treatments are often related to the patients' severity of illness and medical histories.

The GRACE principles (Good Research for Comparative Effectiveness) describe a hierarchy of evidence for observational research on comparative effectiveness that can be used by decision makers, as well as key elements of good practice, including defining research questions and methods; collecting valid, clinically relevant data; analyzing, interpreting, and reporting data; and conducting these studies in accordance with accepted good practices.

The methodological challenges in observational studies of comparative effectiveness primarily stem from the lack of randomization to treatment. This lack of randomization leads to concern about bias and confounding. These can be addressed through study design (exclusion, matching, or restricting the study groups to new drug users) or by analysis (restriction, stratification, or mathematical modeling), but the lack of clarity about the exact methodologies used and

the ongoing debate about the best practices to use can lead to results that are not valid.

The intent of the GRACE principles is to provide guidance for the execution and evaluation of observation studies of comparative effectiveness to enable decision makers to distinguish high-quality research. A key part of the GRACE initiative is creating a Web-based, freely accessible library of real-world case studies that illustrate how observational comparative effectiveness studies can be used to support decision-making for product access, formulary placement, treatment guidelines, or any other decisions relating to approval for payments or adopting use.

The principles offer a set of guided questions that may be a useful guide for observational studies of comparative effectiveness. Three questions represent the GRACE Principles for evaluating non-randomized studies of comparative effectiveness:

- Were the study plans, including research questions, main comparisons, outcome, etc., specified in advance of conducting the study?
- Was the study conducted and analyzed in a manner consistent with good practices, and reported in enough details for evaluation and replication?
- How valid is the interpretation of comparative effectiveness for the population of interest, assuming sound methodology and appropriate follow-up?

Source: The GRACE Initiative, April 2010. For more information, visit graceprinciples.org.

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they don't produce this evidence, someone else will. The information that decision-makers want is not even a comparison of drug A to drug B, but data people need to make decisions. Pharmaceutical companies understand this, but it can be very risky and expensive for them to conduct head-to-head trials. This has led people to ask if there are ways that they can use observational methods. Representatives from the industry estimate that they're going to be tacking on hundreds of millions of dollars to their research development programs for comparative effectiveness for a single drug. This is going to have an impact all the way back to go/no-go decisions earlier in development because companies are going to have to plan not only for getting approval and postmarketing commitments but also the other studies they may need to do. Comparative effectiveness is the new path to marketing a product. Marketing is no longer going to be able to rely just on preapproval trials and vague assertions about how a new product is better in the real world. A formulary committee is going to want to evaluate the dossier on more specific information, particularly for products in a class for which there are available therapies. The committee is going to want to know why a product is more effective than what is currently available. Smart marketing organizations are going to recognize this and put their dollars toward comparative effectiveness.

BLANSETT. KANTAR HEALTH. I don't think it is a pharma company's job, per se, to conduct comparative effectiveness studies. It is their job to demonstrate the efficacy of the products they are bringing to the market. The healthcare reform law is going to work reasonably well, because as an independent organization it is going to set priorities and control the funding. Independence from Medicare and Medicare authorities is a big issue. But pharma companies are, absolutely, going to have to think about how they design their Phase III trials. Companies may need to look to modify later-phase trials where it makes sense to collect additional data to help support comparative effectiveness later on. There will also be a strong argument for post-marketing research, registries, and observational studies. This is already happening in the cancer area where a product can receive accelerated approval with the understanding and agreement that the manufacturer will conduct Phase IV follow-up studies and produce data proving the product is safe and effective in the larger population.

COLUCCI. PUBLICIS. In the end, companies will



Comparative effectiveness is the new path to marketing. Marketing is no longer going to be able to rely on vague assertions about how a product is better in the real world.

DR. RICHARD GLIKLICH

Outcome

do what they need to do to make their medicines available to those who most need them. Maybe it's my eternal optimism, but I think the industry will do the right thing. Every industry has processes that can be performed better, and the pharma industry has shown it is driven by doing things right most of the time. If comparative effectiveness is broadly looked at in the way that it should be, then there is no doubt in my mind that the appropriate use of drugs will prove the best and most cost-effective way to approach illness.

DOYLE. QUINTILES. For marketing, comparative effectiveness is very exciting. Evidence-based marketing gives companies an opportunity to address not just the features and benefits of products but also the drug's real-world performance. In the past, I think marketing was handcuffed to the clinical trial data as the proof of value. Going forward, the clinical trial data are the initial contribution to a pool of evidence. New channels — registries, REMS, Phase IV trials, and other postlaunch activities — will be constructed to continue to create evidence of value for a product. But there is an interesting new challenge: there will be new stakeholders who are more sophisticated and who are demanding customized information about real-world drug performance. Policymakers, payers, patients, and even physicians are now asking for evidence of value.

CROWN. I3 INNOVUS. Comparative effectiveness research will affect the gamut of drug development, starting with who is enrolled in

the trial and how the data are collected. It is widely recognized that the inclusion and exclusion criteria of clinical trials result in study populations that are substantially different from those who end up being treated with the product in the real world. Also, clinical trials measure average treatment effects, often masking variations in response among patient subgroups defined as race, gender, age, medical co-morbidities, and concomitant medications. Treatment options that work better in niche populations may benefit from CER; this is especially true for treatments that have favorable compliance/adherence or side-effect profiles and perform better than alternatives in clinical practice. This focus on overall effectiveness, rather than narrowly defined efficacy, will in turn lead to database studies, registries, and Phase IV trials that are designed specifically to address the limitations of conclusions drawn from meta analyses of the clinical trial literature. Changes are also expected in the tools used to identify patients likely to benefit from a treatment. Without a diagnostic tool to identify likely responders — or screen out non-responders — it will be difficult to effectively wring the costs of non-response out of the healthcare system. A diagnostic tool such as this will allay payer fears of therapeutic creep in the use of expensive biologics to new indications. ♦

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THE REAL WORLD

of Comparative Effectiveness Research

Comparative effectiveness data are geared broadly to multiple decision-makers to provide real-world evidence of the outcomes of various treatments. But challenges remain about developing high-quality data and how the data will be used.

Experts agree that we need a healthcare system that can measure and evaluate real-world performance of various treatments. The U.S. healthcare system ranks 37 among healthcare systems of about 200 worldwide countries ranked by the World Health Organization. The U.S. healthcare system is considered one of the most expensive systems in the world, spending about 16% of its GDP on healthcare yet about 47 million people are uninsured.

Experts also agree that determining outcomes in the real world is challenging.

John Doyle, Dr. P.H., VP and practice leader for the consulting group at Quintiles, points out that the real world is a lot more complicated than the clinical trial world.

"The techniques that have been established over the last 40 or 50 years involving efficacy and safety don't necessarily translate readily to the real-world investigation of performance," he says. "We need to establish best practices for evaluating drug performance in an observational setting. There are a couple of academic groups that have published reports around best practices and observational research but they've been underfunded and under-recognized."

Ken Kramer, Ph.D., senior VP, medical director, at Alpha & Omega, says the challenge in doing comparative effectiveness is going to be defining the endpoints.

"Are we going to have a specific set of endpoints per therapeutic area?" he asks. "Are we going to have endpoints that go across therapeutic areas? The challenge is going to be defining what needs to be evaluated."

The criteria used for measuring comparative effectiveness is going to be important, agrees Nick Colucci, president and CEO of Publicis Healthcare Communications Group.

"As an example, for an oral antibiotic, are we going to look at its spectrum of coverage for a certain group of pathogens or disease states with no regard to how often it needs to be taken or whether concomitant behaviors such as taking it with milk or food or limits on sun exposure?" he asks.

"Payers think of this as 'fluffy' stuff that doesn't matter," Mr. Colucci continues. "But people may not take their medications because it is difficult to comply, and that will affect an outcome. I'm using something simple like antibiotics, but let's think about the complexity of this issue as it relates to blood pressure medicine or other types of medicines that are a lot more complicated, and lifestyle issues become more important, as with diabetes."

Richard Gliklich, M.D., president and CEO of Outcome, says another challenge is increasing the ability to use multi-stakeholder perspectives and defining research questions and research design.

"For example, we're doing a project with the Agency for Healthcare Research and Quality (AHRQ) for uterine fibroid disease, and the first step in that project was to bring together all stakeholders," he says. "That included pharma and device companies, patient and provider organizations, physician groups, researchers, and payers. We went

Comparative Research Priorities

A report released last year from the Institute of Medicine (IOM) recommends 100 health topics that should get priority attention and funding from a new national research effort to identify which healthcare services work best. It also spells out actions and resources needed to ensure that this comparative effectiveness research initiative will be a sustained effort with a continuous process for updating priorities as needed and that the results are put into clinical practice.

A committee convened by the IOM developed the list of priority topics at the request of Congress as part of a \$1.1 billion effort to improve the quality and efficiency of healthcare through comparative effectiveness research outlined in the American Recovery and Reinvestment Act of 2009.

The 100 priority areas reflect the insights of health professionals, consumer advocates, policy analysts, and others who submitted nominations through an online form that was open to any individual or organization and through presentations at public meetings. The committee received 1,268 unique topic suggestions, which it narrowed to 100 based on a set of criteria that included its charge to develop a balanced portfolio. The list reflects a range of clinical categories, populations to be studied, categories of interventions, and research methodologies. The committee developed its list of priorities independent from the comparative effectiveness research activities that other organizations have been charged to do through the American Recovery and Reinvestment Act.

The report also recommends actions necessary to establish an ongoing comparative effectiveness research effort that would not only carry out studies on the 100 recommended initial topics, but also develop priorities for future research and translate the knowledge gained into improvements in clinical care.

Source: Institute of Medicine.
For more information, visit iom.edu.

Consumers Are Skeptical About Evidence-Based Healthcare

There is a fundamental disconnect between the central tenets of evidence-based healthcare and the knowledge, values, and beliefs held by many consumers, according to a recent study by the research group Health Affairs. For healthcare experts, variation — in quality among healthcare providers, the evidence base regarding therapies, and the effectiveness and cost-effectiveness of treatment options — is understood. Yet such concepts are unfamiliar to many Americans.

The study found that for consumers to truly engage in using evidence for decision-making, they have to be informed about the relevant choices for their own situation; value the use of evidence in making those decisions, even if it contradicts conventional wisdom; and accept their role in this process and feel capable and ready to assume it.

Key findings:

- **Misconceptions:** Participants had crucial misconceptions about the underlying concepts of evidence-based healthcare. They found terms such as “medical evidence,” “quality guidelines,” and “quality standards” unfamiliar and confusing. Additionally, only 34% of participants ever recalled having a physician discuss what scientific research had shown about the best way to manage their care. Many participants assumed that their healthcare providers always based decisions

on medical evidence, which to them consists just of “things like my test results and medical history.”

- **Beliefs And Values:** Study participants consistently voiced a number of values and beliefs that were at odds with evidence-based approaches. Although focus-group participants could envision a healthcare provider’s making an occasional mistake, they found it hard to believe that providers could deliver truly substandard care.

Although policy experts define guidelines as best clinical practices based on a large body of medical evidence, focus-group participants perceived them as rigid rules that interfere with providers’ ability to draw upon their medical training and experience to tailor their care to the characteristics of individual patients.

Participants also believed that any new treatment is improved treatment. This attitude may help explain the survey finding that only 47% of respondents agreed that it is reasonable to pay less out of pocket for the most effective treatments and drugs. Linking cost sharing to clinical effectiveness may be perceived as restricting treatment options, particularly for unproven therapies.

- **More Costly Care Is Better:** A substantial portion of focus group and interview participants expressed the view that “you get what you pay for.” A third (33%) of survey respondents agreed or

strongly agreed with the statement that “medical treatments that work the best usually cost more than treatments that don’t work as well.” Although 27% disagreed or strongly disagreed, 40% reported that they were not sure about this.

- **Behaviors In The Medical Encounter:** The survey results indicate that many consumers do not engage in behaviors that could be beneficial to them during medical encounters. More than half of the respondents had never taken notes during a medical appointment (55%) or brought online information to discuss with their doctor (60%). Almost half had never brought someone to provide support or advocacy (44%). In addition, 28% of the respondents had never brought questions to ask their doctor.

Patients often rely heavily on their doctors for information, interpretation, and guidance on treatment options. Thus, they may be reluctant to question or challenge what the doctor advises. In the survey, 41% of respondents reported that they had not asked questions or told their doctor about medical problems, because the doctor seemed rushed or they were unsure about how to talk to him or her.

Source: Health Affairs.

For more information, visit content.healthaffairs.org.

through a process of prioritizing research questions among this broad stakeholder group. This is a new process that is going to have to be added onto these research designs. It takes time, but it is very valuable.”

Dr. Doyle says everyone needs to come to terms with the fact that doing high-quality comparative effectiveness is going to be very difficult.

“Confounding and bias are two threats to validity, and we have done a good job of addressing those in the clinical trial setting through randomization and control,” he says. “We lose those tools to a great extent in CER. That means we need to become very adept at using new tools to control for bias and confounding. Otherwise we run the risk of cap-

turing a lot of attention and headlines in launching CER studies but then undermining the ability to evaluate comparative effectiveness because we’re not doing it in a valid and reliable manner.”

Dr. Doyle adds it may take years for researchers to determine good comparative effectiveness research practice, something analogous to GMP and GCP.

“There is a need for guidelines, and the Patient Centered Outcomes Research Institute (PCORI) would promulgate best practices,” he says. “There is a need for a clearinghouse for this type of research. This observational research is different from experimental research, and we need to be sensitive to this when designing and interpreting stud-

ies; they run across a whole spectrum of design.”

There needs to be some convergence around what regulators and what payers are asking for with regard to drug performance evidence, Dr. Doyle says.

“The FDA wants more information on the long-term data on a product’s safety profile,” he says. “I would argue that the agency hasn’t asked enough question about the complement of benefits in conjunction with safety queries to get a more holistic view of risk/benefit. And once this world of risk/benefit is brought to light, we begin to realize that this is very closely aligned and linked to cost/benefit. By linking up the two, we can get a better view of real-world performance of a drug.”

Comparative EFFECTIVENESS

Using CER

Bill Crown, Ph.D., president of i3 Innovus says eventually CER will touch every aspect of the healthcare continuum.

“For many conditions, there is a great lack of guidance for physicians around which treatments should be provided to which patients,” he says. “To reduce the variance and produce guidelines for physicians, the industry needs to set an agenda that examines how treatments compare with each other. Indeed, many biopharmaceutical and medical device companies already are building comparative effectiveness research into their clinical development programs to generate data that will show the value of their products to payers by the time they receive FDA approval. They know that to get ahead of the curve and get maximum reimbursement, they have to incorporate both the clinical response and the real-world implementation associated with their treatments versus another.”

Larry Atkins, executive director, U.S. public policy at Merck & Co., says comparative effectiveness should be a tool to support decision-making.

“Nobody’s interests are served by poor-quality decision-making,” he says. “If we have good-quality comparative effectiveness information, we should be using it in high-quality decision-making structures. The difficulty is that we don’t have a system for making decisions about how to allocate resources that is disciplined to the scientific evidence. This requires both a high-quality comparative effectiveness system that is turning out high-quality reviews and a system that is well-disciplined in making decisions about allocating resources that is true to the science.”

Lee Blansett, senior VP of Kantar Health, says the language of the healthcare reform law precludes comparative effectiveness findings from being construed as explicit coverage policies.

“But that doesn’t mean the findings can’t be used to inform coverage policy,” he says. “It would be unnatural and unlikely for Medicare and commercial payers not to try to find some way to use that information to guide coverage. In Europe, comparative effectiveness is start-

ing to bleed into coverage, and I fully expect that will also happen in the United States.”

Mr. Colucci says CER will be used in consideration as to the price and the reimbursement of the medication.

“Many people who were working on this are legitimately trying to say it’s not all about cost,” he says. “But to suggest CER has nothing to do with cost would be disingenuous.”

A Look Outside the U.S.

Susanne Michel, M.D., head of market access, Europe, at Kantar Health, says comparative effectiveness was a consideration for the World Health Organization beginning in

2002, when it called upon countries to establish a policy starting with vaccination strategies.

“Today, NICE (the National Institute for Health and Clinical Excellence) gives a lot of statements around comparative effectiveness because as a public funder and provider of healthcare it needs to think about that,” she says. “In Europe deciding the effectiveness is based on cost and on the basis of a technology’s or drug’s utility. NICE in the United Kingdom is an example. In Germany, a utility approach is being developed. CER studies begin to creep in during decision-making or in scientific advisory meetings, where decision-makers will be able to weigh the benefits across different patient populations.” ♦

Health Economics Spending Increases 45% in Emerging Markets; 8% in the U.S.

Pharmacoeconomics spending is increasing by 8% this year in the United States, but winning company buy-in remains a challenge for many health economics teams, according to a new study by Cutting Edge Information.

Sixty-six percent of surveyed respondents across all life-sciences sectors reported having higher spending levels, while 27% expected spending to stay the same, and only 7% reported a decrease. Health economics spending in Europe and Canada increased 21% and emerging markets have seen a 45% increase in funding, according to the new study, *Health Economics and Outcomes Research: U.S., Europe, Canada and Emerging Markets*.

“Rising budgets for health economics and outcomes research makes sense, as payers’ requirements are becoming increasingly complex and stringent,” says Jason Richardson, president of Cutting Edge Information. “Companies also use the data to inform portfolio decisions throughout drug development and commercialization.”

The growth in emerging markets underscores the industry’s desire to expand capabilities in these regions. Small pharmaceutical and biotechnology companies made the largest increases in their pharmacoeconomics investments, with average spending growth of 55%.

Large and mid-sized drugmakers also showed a smaller, but healthy growth rate of about 15%. Medical device companies’ average spending on health economics has remained flat.

Research findings emphasize that teams must continue working to achieve buy-in and prove value, a task especially difficult for a specialized function that often must use economically complex vocabulary to communicate long-term value. Project participants identified communication with internal and external clients as a key challenge because stakeholders bring widely varying perceptions about the value of pharmacoeconomics.

Source: Cutting Edge Information. For more information, visit cuttingedgeinfo.com/health-economics.

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