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Comparative Effectiveness Research: Opportunities and Analysis

**Perspectives from
Disruptive Women in Health Care**

Comparative Effectiveness Research Smack Down: June 22nd Kicks Off A Policy Challenge through the Eyes of Disruptive Women in Health Care

Health reform fever is spreading throughout Capitol Hill, and up and down Pennsylvania Avenue. Among the topics consuming the health intelligentsia is comparative effectiveness research (CER). (For examples, click [here](#) or [here](#).)

AHRQ, [The Agency for Healthcare Research and Quality](#), has defined CER as:

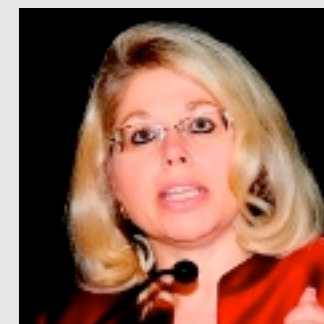
A type of health care research that compares the results of one approach for managing a disease to the results of other approaches. CER usually compares two or more types of treatment, such as different drugs, for the same disease. CER can also compare types of surgery or other kinds of medical procedures and tests. The results are often summarized in a systematic review.

[The American Recovery & Reinvestment Act](#) (ARRA) allocated \$1.1 billion for comparative effectiveness research. It also established a 15 member [Federal Coordinating Council](#).

Like most things in health policy, CER is more complex than it may appear at first blush. While supporters herald its ability to save money and provide information on the most appropriate treatments for patients, others raise a number of cautions that policymakers and legislators would be wise to consider.

Given the multi-faceted nature of this issue, I have reached out to

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President & CEO,
Amplify Public Affairs;
Founder, Disruptive
Women in Health Care



Robin Strongin is an accomplished public affairs expert with more than 25 years of experience working in Washington, DC. Her areas of specialization include health care, science, technology and innovation. Robin has worked with and for Federal and state governments, regulatory agencies, Congress, think tanks, nonprofit organizations, corporations, coalitions and trade associations. Robin is currently serving on the Public Affairs Council's Senior Executive Task Force and the AcademyHealth Health Policy Communications Interest Group Advisory Committee. She has recently been appointed to the board of the Juvenile Diabetes Research Foundation (JDRF).

several of our bloggers to write about the topic, each from their unique perspective and area of expertise. While much has been written about the benefits of CER, we wanted to remind our health policy brethren to address some of the thorny issues.

We look forward to hearing your thoughts as we wrestle with this particular health policy challenge.

What is Comparative Effectiveness?

“As applied in the health care sector, an analysis of comparative effectiveness is simply a rigorous evaluation of the impact of different options that are available for treating a given medical condition for a particular set of patients. Such a study may compare similar treatments, such as competing drugs, or it may analyze very different approaches, such as surgery and drug therapy. The analysis may focus only on the relative medical benefits and risks of each option, or it may also weigh both the costs and the benefits of those options. In some cases, a given treatment may prove to be more effective clinically or more cost-effective for a broad range of patients, but frequently a key issue is determining which specific types of patients would benefit most from it. Related terms include cost–benefit analysis, technology assessment, and evidence-based medicine, although the latter concepts do not ordinarily take costs into account.”

... “A recent example of a comparative effectiveness study indicates that careful analysis can sometimes disprove widely held assumptions about the relative merits of different treatments. The study, which involved patients who had stable coronary artery disease, compared the effects of two treatments: an angioplasty with a metal stent combined with a drug regimen versus the drug regimen alone.⁵ Patients were randomly assigned to receive the two treatments, and although the study found that patients treated with angioplasty and a stent had better blood flow and fewer symptoms of heart problems initially, the differences declined over time.⁶ More importantly, it found no differences between the two groups in survival rates or the occurrence of heart attacks over a five-year period.”

From “Research on the Comparative Effectiveness of Medical Treatments,” Congressional Budget Office, 2007.
<http://www.cbo.gov/ftpdocs/88xx/doc8891/12-18-ComparativeEffectiveness.pdf>

Comparative Effectiveness Research: What a Difference an X Should Make

With all the chatter and perhaps now “twitter” about health care reform, one area has gotten a lot of attention and it is an issue that is near and dear to the [Society for Women’s Health Research](#)—that is comparative effectiveness research (CER).

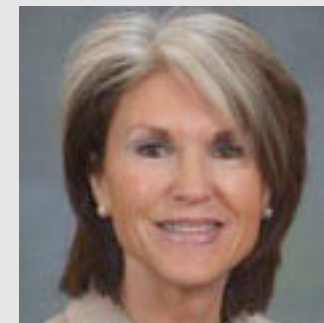
Since it is accepted knowledge that women and minorities were not, and are still not, to the degree they should be, included in clinical trials, there is much we are learning and still do not know about sex and ethnic differences in terms of prevention, diagnosis and treatment. My concern with CER, therefore, is how differences in effectiveness and treatment will be determined. Several genetic, hormonal, environmental factors influence health and disease in particularly different ways in women and men. Because of that, CER must study both men AND women and analyze fully any sex based difference in disease prevalence, treatment options and procedures. Those decisions must correlate to real world experiences.

In January, I [sent a letter to Senator Tom Harkin](#) that outlines the Society’s concerns and offers suggestions and recommendations.

CER decisions must be clinically relevant to decisions made by health care professionals and patients and that to achieve this, patients must be provided a voice in the health care decision-making and determination process.

The research results must avoid creating unintended access

Phyllis Greenberger
President & CEO,
Society for Women’s
Health Research



Phyllis E. Greenberger, M.S.W., is frequently called upon to speak on topics such as the importance of the inclusion and retention of women in clinical trials and the importance of sex-based biology and gender-based medicine. She serves on many boards of directors, advisory and editorial boards, and was a member of the Research Committee of the Presidential HIV/AIDS Advisory Council (PACHA) and the National Institutes of Health’s National Advisory Environmental Health Sciences Council (NAEHS). Currently, she serves as the consumer representative to the Medicare Coverage Advisory Committee (MCAC) for the Centers for Medicare and Medicaid Services (CMS) and has been a consistent advocate for coverage for new technologies.

barriers, coverage denials, or arbitrary dollar thresholds that can arise through centralized cost effectiveness determinations. By focusing on gaps in clinical knowledge the research findings may improve the quality and ultimately, the value of health care.

I am encouraged that most recently there has been attention to this issue and that many of the concerns raised by the Society and other groups are being discussed and in some pieces of legislation are addressed. However, the devil is always in the details and there are several legislative proposals out there and it is not clear which one or which parts will prevail and when and by whom these decisions will ultimately be made.

As an organization whose mission is to improve the health of all women through advocacy, research and education, we will keep a watchful eye on this and other issues that may have negative consequences for women in this health care reform debate.



Comparative Effectiveness Research: Thinking outside the box

“\$2.5 Billion spent: no alternative medicine cures” screamed the [headline](#) two weeks ago. “You expect scientific thinking” one expert was quoted as saying, claiming that it’s become “politically correct to investigate nonsense.”

So what’s the real issue here? Better yet, is there a way to bridge the gap between Western and Eastern philosophies so that the constituent that matters most in this paradigm — the patient — wins?

I believe that when it comes to comparative effectiveness research (CER, i.e. the efficacy/superiority of one drug or modality compared to another), the heart of the West vs. East battle is two-fold and until we find ways to overcome philosophical barriers, never the twain shall meet.

First, we must examine the funding factor. Notably, most Western studies have been and continue to be privately funded and simply fade away with little fanfare if findings are negative or inconclusive. Conversely, a majority of studies that examine “unconventional” or alternative treatments have had the taxpayers footing the bill. Consequently, let’s ask what we can do to engage institutional and pharmaceutical interests so that the funding conundrum is more equitable?

Second, (and more importantly) are Western researchers attempting to fit a square peg into a round hole? Does ‘one size fits all’ work? Eastern research has long been based on an

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inductive method that relies on direct observation of the individual and his/her relationship to environmental insults. On the other hand, Western research is reductive (or deductive) with general observations evolving into a statistical design that leads to certain conclusions. Although there has been a movement within the Eastern research community to incorporate traditional Western methodologies (e.g. control groups, randomization) one must wonder if by doing so (and thereby eliminating the subjective element that has driven data collection for centuries) the modality under investigation becomes diluted in terms of quality of practice?

Rather than screaming “foul” and “nonsense,” shouldn’t we be encouraging innovation in scientific methodology and CER in ways that integrate Eastern inductive approaches into Western reductive strategies so that the researchers can truly measure efficacy? A new paradigm is a win-win for all.

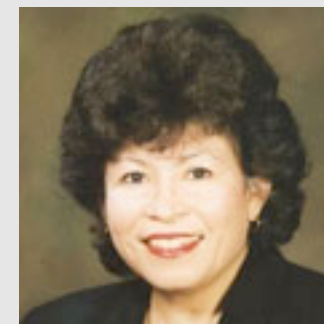
Comparative Effectiveness Research Can Help Combat Health Disparities

My organization, the National Hispanic Medical Association, is committed to improving the health of Hispanics and other underserved. We support policies that will reform public health and medical services to decrease health care disparities and improve the health status of vulnerable groups. The National Disparities Report by the Agency for Healthcare Research and Quality found that our community has the worst access and quality care compared to non-Hispanics in the nation.

Evidence-based public health and medicine strategies are necessary to decrease variation of service delivery that impacts and rations care to Latinos, especially in our poor neighborhoods. Medical treatment should be based on comparative effectiveness value of treatment strategies that produce the greatest benefit for the Hispanic community at the lowest cost. We recognize that comparative effectiveness research is about value in health care. We also heed the concerns of those who have warned to beware of a “one size fits all” approach that could decrease access to treatments for minority patients. However, NHMA believes that comparative effectiveness research will add to the body of knowledge for reducing health disparities for 1) physicians to use to improve quality care for patients; as well as 2) for administrators to use to improve health systems for delivery in following priority areas:

1. Cultural competence and health literacy research in order to ultimately change behaviors and improve lifestyle in our communities

Elena Rios,
President & CEO,
National Hispanic
Medical Association



Dr. Rios also serves on the National Hispanic Leadership Agenda, Campaign Against Obesity, and the Partnerships for Prevention Boards of Directors, the American Medical Association Commission to End Health Disparities, the PacificCare-UnitedHealthcare California Investment Committee and is Chair, the National Coalition on Hispanic Health. Dr. Rios has lectured and published articles and has received several leadership awards, including awards from the U.S. Department of Health and Human Services, the Congressional Black, Hispanic, Asian and Native American Caucuses, American Public Health Association Latino Caucus, Association of Hispanic Health Executives, Minority Health Month, Inc., Hispanic Magazine, Verizon’s First Pollin Community Service Award, and Amerimed.

2. Effective ways of communicating with Hispanic patients and their families
3. Knowledge about health disparities interventions between hospital systems and clinics that have longstanding experience with Hispanic physicians and their patients
4. Innovative research targeted to Hispanic patients and their families
5. Integrated case that is outcomes based – and with mental health and oral health as well as physical health

Possible Responses by Private and Public Insurance Plans

“To affect medical treatment and reduce health care spending, the results of comparative effectiveness analyses would ultimately have to change the behavior of doctors and patients—that is, to get them to use fewer services or less intensive and less expensive services than are currently projected. Bringing about those changes would probably require action by public and private insurers to incorporate comparative effectiveness information into some combination of their coverage and payment policies. Those steps, however, could be difficult and controversial.”

... “Making substantial changes to insurance design and reimbursement would not be easy. Some patients, providers, and other interested parties would probably object to such arrangements or to the manner in which insurers established them. A particular concern would be that the average effects found by studies might not apply to all types of patients that were considered—so that sub-groups of patients who could benefit greatly from a treatment might be overlooked. And as discussed above, having research studies keep pace with the development of new technologies would be an ongoing challenge. Consequently, any new incentive systems would probably be applied only in areas of care where the evidence was convincing.”

From “Research on the Comparative Effectiveness of Medical Treatments,” Congressional Budget Office, 2007.
<http://www.cbo.gov/ftpdocs/88xx/doc8891/12-18-ComparativeEffectiveness.pdf>

Comparative Effectiveness Research from a Caregiver's Perspective

Caregivers need all the help we can get in navigating the confusing and often conflicting health, medical, and financial decisions required to support a family member with severe or chronic health conditions. My husband, Paul Berger, suffered a severe stroke from a ruptured aneurysm at the young age of 36. Our insurance covered his acute medical needs—brain surgery, hospital care, and even some rehabilitation services. Reimbursement ended when he was discharged from the rehab unit of the hospital, but he still needed—and could benefit from—long term speech, occupational and physical therapy.

The first decision was how to continue care. Should we try to coordinate outpatient care through the hospital, or through independent practitioners? Should we sacrifice our savings by spending a lot of money to extend his inpatient time at the hospital or transfer to another less costly one nearby? Should we sacrifice our time together and support of local family and friends to send him out of town to the state's only comprehensive rehab facility run by the department of vocational rehabilitation services a few hours away?

In my professional life, and intellectually, I strongly supported community-based care. My gut told me that Paul needed to stay connected in town, and find a way to make it work from home. My heart didn't want us to be separated. I became Paul's "case coordinator" and orchestrated his weekly therapy schedule, periodic doctor's visits, paperwork, prescriptions, equipment, home modifications, a daytime driver/companion/homework

Stephanie Mensh



Stephanie Mensh found herself thrust into the role of personal caregiver and patient advocate in the same shocking instant that her husband, Paul Berger, suffered a severe stroke. That was over 20 years ago. Since then, they have both thrived due to teamwork and Stephanie's caregiving mantra: push the survivor to be as independent as possible; do what you (the caregivers) do best; and communicate. To help other caregivers, persons with disabilities and their families, Stephanie serves on the consumer board of the newly established Stroke Comeback Center in Oakton, VA, and served on the Steering Committee of the National Quality Forum's project on Stroke Measures as a consumer representative. Stephanie has co-authored books on stroke recovery with her husband. She works with Paul in their publishing company and contributes to their website on stroke recovery, www.strokesurvivor.com.

helper (since I continued to work full time), vocational rehab assessments, contact with his employer, and many of the chores Paul had handled—taking care of our finances, among others.

This was twenty years ago. At the time, AHRQ's predecessor agency had the ambitious goal of writing clinical practice guidelines for major conditions affecting Medicare's budget. Cataract surgery and treatment of benign prostate enlargement were at the top of the list. Post-stroke rehabilitation made it into the mix, and Paul and I testified during a public hearing. We also coordinated testimony by speech and physical therapists to talk about the need to clinically recognize that stroke survivors – like Paul – could continue to make progress many months, indeed years, after their stroke.

These guidelines are in the archives at AHRQ. A few years ago, the Department of Veterans Affairs developed a set of post-stroke rehabilitation guidelines. I have seldom, if ever seen these guidelines provided to survivors and their families.

I believe that for comparative effectiveness research (CER) to be successful, meaningful public outreach is needed to get the information into the hands of the patients and their families. CER needs to provide advice for real life decisions in real time. Studies must be designed with the patient in mind. Myrl Weinberg, President of the National Health Council, said it best:

“Comparative effectiveness research should supply us with good evidence and data about what works and what doesn't. However, it should not be just about one product against another, or treatment process, or combination thereof. We also need to look at how different delivery systems can be compared.” www.nationalhealthcouncil.org

Comparative Effectiveness Research: Through the Lens of Medical Innovation

The Top headline of FDA News Device Daily today read, “Comparative Effectiveness Research has Benefits, Risks Experts Say”. Why would Device Daily consider comparative effectiveness to be risky? Many obvious concerns come to mind. There are distinct risks that the **process** imposed on the device industry may stifle growth. Worries abound related to the direction policy makers may employ such as **when** studies will be required (e.g., at the early stages of development, or later in the cycle of real-world experience), **how** studies will be conducted (e.g., by the government or a public/private entity), **who** will determine the type, scope, design and rationale for conducting such studies, **what** the studies will be used for (e.g., to restrict coverage, to control access).

The medical device industry has legitimate reason to worry from the increasing banter of politicians and inexperienced policy makers suggesting that comparative effectiveness controlled by a new government agency will be the silver bullet to manage unbridled utilization of medical interventions. Those experienced in device trial design and commercialization strategies for devices know that the struggle to move new innovation to the patient already takes years driven by inconsistent and irregular evaluative processes imposed by technology assessment authorities in some 3000 private payer health plans, individual hospital technology assessment groups, state Medicaid coverage entities, and Medicare local and national coverage processes. Adding another evaluative process, an entity to conduct

Randel Richner,
Founder & President,
Neocure



Randel Richner founded Neocure Group in June 2006, a consulting firm specializing in reimbursement, health policy, economics, and government advocacy support for biopharmaceutical and medical technology companies. Prior to founding the Neocure Group, Richner worked for Boston Scientific Corporation and GlaxoSmithKline. She served two terms as the first industry representative to the Executive Committee of the Medicare Coverage Advisory Committee. Richner is a frequent contributor on policy panels on health care issues related to the technology industry. Before her career in health economics and policy, she was a practicing dialysis and transplant nurse for 13 years at the University of Michigan Hospital and Northern Michigan Hospital.

comparative evidence research, rightly gives technology manufacturers who are currently subject to multiple assessments reason to question the true utility of another over-arching authority.

To seriously understand why and how procedures are or a technology is properly used (and is it cost-effective?) one must examine how technology use (or underuse) is linked to the payment process. Few policy makers understand the complicated payment system for medical technology that rewards doctors for the most acute, lengthy, or complex procedures performed in the highest priced site of service. **Until we correct the arcane formulas that punish any technological advance that is simpler, more efficient, and delinked from physician time, any “comparative effectiveness” study is irrelevant.** To recoup the extraordinary costs of development, manufacturers and physicians often have no choice but to seek fair payment through complicated coding processes that are completely disassociated from the true value of a device or procedure.

Innovators need a crystal ball to commercialize. The investor community, who fuels the pipeline of great new technological advances, has serious concerns about how the new comparative effectiveness entity may affect the future, especially the amount of investment it will take to bring something promising to commercialization. New investors, particularly private equity and hedge funds, are likely to severely curtail device investing due to the uncertainty of the expected increased development costs or delays to market.

So, Is comparative effectiveness a rational approach to managing utilization and costs?

Indeed, in principle, we need more and improved information to ensure we are wisely using resources. Randomized controlled trials (the foundation of comparative effectiveness research) have a role in large target populations, where making the wrong recommendation is high. In addition, it is important to model the risk and potential consequences of making the wrong recommendations. Failure to develop a rigorous, systematic approach to evaluating tests and interventions can lead to mistakes—good tests for instance may fail to be adopted, and bad tests may be the norm. Be wary of the manufacturers that argue RCT’s are not feasible under most circumstances. Nonetheless, we need a new paradigm for evaluation that is transparent, independent, rigorous and scalable.

The era of “trust me” science is over. We must more directly rely on the good science and studies generated by the FDA—the pre-IDE process—the very important and time-tested process – and acknowledged as valuable by all parties. Use

new study approaches developed at the FDA to inform decision making and increase the rigor and scalability of these methods and apply to “comparative effectiveness” research.

In the words of Henry Waxman, Chairman of the House Energy and Commerce Committee, “We need to compare whether some of these new ideas are really advances or more costly alternatives to what we know will work”. Medical innovators with products that improve patients’ lives passionately embrace proving their worth.

A fair, open, cooperative public/private process, if designed properly will enhance the information needs of patients, physicians, and scientists and eliminate wasteful and ineffective medical technologies and procedures. Implemented without a fix of the payment system, however, the fear that this may stifle access and innovation will be realized.



Comparative Effectiveness: Dance time for rare and genetic diseases

I've been tasked with presenting the genetic and rare disease perspective on comparative effectiveness.

I'll dispense with 'rare' right away. If by rare, we mean single gene disorders, then perhaps it is a useful designation.

If by rare, we just mean the equivalent of the US definition of orphan disease, i.e., less than 200,000 people in the US, then we should ask a few questions. In the old model of test and drug development, the 'block buster, body count', model, rare was a useful designation. In a system built for BIG, then rare needed a boost. In the new age of personalized medicine, all conditions are rare. In fact, they are usually an N of 1 after factoring in the myriad of genes involved, epigenetics, environment and so on. As we enter new ways of dealing with common conditions, they too will be fragmented into dozens, sometimes hundreds, of rare conditions. Thus rare and common conditions have similar challenges in that realm. For these reasons, I recommend we lose the word 'rare'. I know it has a rich and abundant history in the Orphan Drug Act, but in addition to the aforementioned issues, I think siloing our thinking around disease into these social constructs of abundance of disease misses opportunities that would blossom were we to consider gene families, pathways and targets instead of incidence and prevalence.

Let's move then to single gene disorders. Comparative effectiveness would have to go on unemployment if it depended on single gene disorders for its first tasks. Most single gene

Sharon Terry
President & CEO,
Genetic Alliance



Sharon Terry is also the founding Executive Director of PXE International, a research advocacy organization for the genetic condition pseudoxanthoma elasticum (PXE). At the forefront of consumer participation in genetics research, services and policy, Ms. Terry serves on many major governmental advisory committees on medical research, including as liaison to the Secretary's Advisory Committee on Heritable Disorders and Genetic Diseases in Newborns and Children, and the National Advisory Council for Human Genome Research. Ms. Terry is committed to bringing together diverse stakeholders that create novel partnerships in advocacy; integrating individual, family, and community perspectives to improve health systems; and revolutionizing access to information to enable translation of research into services and individualized decision making.

disorders do not have a treatment, let alone several. And once one is developed, it is hard for a second one to be developed given the lack of financial incentives in the old paradigm. Perhaps a first step in comparative effectiveness for single gene disorders is creation of ANY treatment for these conditions.

On to genetic conditions. I think genetic conditions have already been spoken for in all of the preceding posts – because all disease is a mix of genes and environment, and so all of the conditions spoke about, either explicitly or implicitly, are genetic to some extent. That said, I believe genetic diseases, and genomic signatures of attributes of disease, like tumors, offer ways to quantitatively measure expression thereby offering a new level of scientific scrutiny for disease. While most genetically and genomically authored tests and treatments are nascent and have not yet been scrutinized, we are seeing some assessments of genetic tests, at least. These are not complete comparative effectiveness studies, but they use methodology that might offer something to the field of comparative effectiveness. If the field does use methodologies like those of [EGAPP](#), then it will be important to do a broader assessment of the methodology before wide spread implementation. From the website, “The project’s goal is to establish and evaluate a systematic, evidence-based process for assessing genetic tests and other applications of genomic technology in transition from research to clinical and public health practice.” There are those who ask why almost all tests that EGAPP has assessed have failed to meet the requirements to pass into clinical practice, when some of these tests have done so in a variety of ways. EGAPP is a good example of how hard it will be to do comparative effectiveness, since its assessment (far from comparative effectiveness) is so difficult, resource and time consuming.

However, I look forward to our foray into comparative effectiveness. I believe it is time for the practice of medicine to move from being a cottage industry. Comparative effectiveness, coming of age in the genomic/informatics era, while we move toward individualized medicine (commonly known as personalized medicine), will be an effective tool if, in the words of the post by Randel before me:

“A fair, open, cooperative public/private process, if designed properly will enhance the information needs of patients, physicians, and scientists and eliminate wasteful and ineffective medical technologies and procedures. Implemented without a fix of the payment system, however, the fear that this may stifle access and innovation will be realized.” If we wish to move medicine out of the cottage industry realm, reimbursement has to come too. There are exciting months ahead of us – let’s not call each other names like rare or common, genetic or infectious, popular or unpopular. No disease, condition, or disorder should be left standing around the edges of the dance floor while the rest are dancing.

Stay tuned to the collaborative discussion about health reform resulting from the flash mob on June 17, 2009.

[Inform Health Reform ; http://fastercures.blogspot.com/2009/06/fastercures-joins-healthcare-advocates.html](http://fastercures.blogspot.com/2009/06/fastercures-joins-healthcare-advocates.html)

Medical Registries

“Another option that could supplement or help improve analyses of claims data would be to establish medical registries, which generally track patients who have a particular disease or who have received a specific treatment. Registries collect additional information that is typically not contained in claims records, such as measures of health status or test results. In the United States, a number of registries—established or managed by various entities, including medical specialty societies and product manufacturers—have been used to help determine the clinical effectiveness or cost-effectiveness of various products and services.⁴⁵ Some health plans establish registries of their enrollees, although a centrally managed registry would have the advantage of being able to track patients if they moved or changed health plans.

“Data from medical registries could help improve claims-based analyses both by allowing a broader set of outcomes to be measured and by providing information to control for differences among patients getting different treatments, including the severity of their illness. But a number of challenges and trade-offs would exist. One issue would be how to recruit patients and their providers to participate in and provide information to the registries and to retain them over time. Voluntary participation might be easy to implement but could introduce bias into analyses if patients choosing to participate differed in important ways from patients who had opted out. Some form of mandatory participation could avoid that problem but might raise objections from participants. Registries focused on specific treatments could also be subject to bias if those patients differed systematically from patients who did not receive those treatments—a problem that could be addressed by including a comparison group in the registries. Another trade-off concerns the data elements to collect; a more extensive list would permit richer analyses but would raise the burden of participation.”

From “Research on the Comparative Effectiveness of Medical Treatments,” Congressional Budget Office, 2007.
<http://www.cbo.gov/ftpdocs/88xx/doc8891/12-18-ComparativeEffectiveness.pdf>

Comparative Effectiveness through the Policy Lens

I don't think we in health policy really understand how complex our systems are and what manner of challenges – some of which we created – present themselves in clinical care.

It was Dr. Christine Sinsky who enlightened me. She said that just five years ago, a patient with a 143/82 blood pressure, 1.4 creatinine, 128 fasting blood sugar and 189 cholesterol was advised to exercise and lose weight. Today, that same person has hypertension, stage 3 kidney disease, diabetes, hyperlipidemia and is a candidate for four medications and ongoing monitoring.

Citing data from the *New England Journal of Medicine*, she added that a Medicare patient with diabetes, hypertension and depression with a complaint of headaches is subject to 56 different quality measures.

The complexity of those – and other – clinical situations is something she manages with smart system integration; no doubt you've seen her publications on those methods. I can't help but wonder if CER – not as it is imagined and promised, but as it will be realized – will help or hurt clinicians like her and patients like hers. As a policy maker of longstanding, I have to admit that we generally had the best of intentions, but always created negative unintended effects.

Will we do that again? Have good intentions but negative effects? Only time will tell. Since all of us will soon be walking the territory,

Glenna Crooks,
Founder & President,
Strategic Health Policy
International, Inc.



Glenna Crooks solves some of the toughest health care problems of our times by distilling chaos and complexity into recognizable and easily digestible, action-oriented insights. Her clients, businesses and governments around the world, have used her Centricity Principle™ approach to create successful organizational, national and global transformational strategies. Her work is based on a professional history in senior government positions as a Reagan appointee, lobbyist and professional society and bio-pharmaceutical company executive. She served on the Bill and Melinda Gates Foundation Pediatric Dengue Vaccine Initiative Board of Scientific Councilors and was a member of the Institute of Medicine committee to advise the Department of Defense on bioterrorism countermeasures.

I'm offering the beginnings of a map in this blog; a map intended to navigate the territory better. I look forward to the ways others might add to our collective understanding of the landscape. The map is not the territory, as they say, but I believe that the better we explore it now, the better will be the chance that those who traverse it can avoid Donner Pass scenarios.

I currently see 7 components on the CER Map, and each of those has a set of interrelated policy issues. I'm interested if you see the territory differently and if you have components and policy issues that illuminate this endeavor further. As you do, remember Dr. Sinsky and Donner Pass.

The 7 components on the CER map:

1. The types of **players** who, as stakeholders or vendors, will be involved:

- * Public sector; e.g., AHRQ, CMS/QIOs, FDA, NIH, VA/DoD, State Governments
- * Private sector, including for-profit groups; e.g., Leapfrog, NBCH, NBGH, NCQA, PBMs, Health plans, insurers, [URAC](#), JACHO, medical centers, producers of products and services (biopharmaceuticals, devices, games), CROs
- * Public-Private Joint Groups; e.g., NQF, OHSU Center for Evidence-Based Policy
- * Foundations; e.g., RWJ, CHCF
- * Academics, professional societies and study groups; e.g., Institute for Clinical and Economic Review (ICER), [American College of Physicians](#) (ACP), [Drug Effectiveness Review Project](#) (DERP), NY Academy of Medicine Evidence Based Medicine Resource Center, IOM, Rand
- * Consumers; e.g., Patient advocates, prescribers, professional societies
- * Global Influencers; e.g., NICE.

2. The types of **targets** that will be deemed important for CER studies:

- * Prevention; e.g., primary, secondary, tertiary
- * Diagnosis; e.g., pt/caregiver report, lab assessment, imaging
- * Condition-related; e.g., all hypertension control methods singly or in combination
- * Treatments; e.g., high cost-low volume, high volume-low cost
- * Chronic care and maintenance

- * Palliative or supportive care; e.g., anti-nausea drugs used with chemotherapy
- * Health care delivery system options; e.g., office care vs. telemedicine, physician vs. physician extender.

3. The types of **evidence** that will be used to conduct studies:

- * Economic/claims analyses
- * EHR records/data mining
- * Clinical guidelines
- * Expert opinion
- * Registries
- * Practical clinical trials
- * Predictive modeling.

4. The preferences for types of evidence along the **traditional hierarchy**:

- * Meta-analyses of individual patient data
- * Large multi-center RCT
- * Meta-analyses of group data
- * Small, single-site RTCs
- * Prospective cohort studies
- * Case control, retrospective cohort or cross-sectional studies
- * Poorly controlled studies
- * Uncontrolled studies
- * Inductive, observational studies.

5. The possible **uses** of the studies:

- * Decision support by clinicians and patients
- * Educational for clinicians and patients
- * Coverage and payment determinations
- * Marketplace entry clearance through product licensure, accreditation or contracting

- * Pay for Performance
- * Evidence-based clinical guideline updates
- * Quality report cards
- * Consumer reports.

6. The **communication methods** by which results will become known:

- * Federal agency publications
- * Professional society peer-review journals
- * Coverage bulletins
- * Web-sites
- * Lay press.

7. Results **implementation** and **outcome expectations**:

- * Implementation; e.g., next insurance contract year, first day of next quarter
- * Outcome expectations; e.g., price reductions, lower costs of care per episode or course of illness, cost trend decline, patient satisfaction, declines in mortality, improved quality of life

Each of those 7 components has different, but clearly interrelated, policy questions, of which the following are but a sample.

Player-related policy questions:

- * Are the players all on equal footing, or are some more important than others?
- * Must players who control major segments of the health care market meet different standards of behavior regarding target selection or evidence type because their decisions are more consequential than the role that others might play?
- * Are the players who pay for larger shares of the health care budget more important than the players who do not?
- * How can the interests of the various stakeholders be accommodated when so many of those are – and will always be – conflicting?
- * How will studies account for the reality that stakeholders each defined value in different ways?

* Who will be allowed to conduct useable CER studies? Will credentialing be required of individuals and/or organizations that do CER studies? That is, can anyone with a laptop and data generate a CER, or must they be somehow certified or licensed to do so?

* Can companies do CER studies of their own products in comparison to other modalities (e.g., statin vs. oatmeal)? If so, can these studies be used to change product labels and promotional approaches?

* Can patients opt out of data bases on which CER is conducted? If so, what are the consequences for their care and the validity of the data?

* How will patient outliers be protected? What if patients are harmed by the results? Who is accountable in the chain of those who study, communicate and make decisions based on the results?

* Will there be exceptions or exclusions for rare diseases, and for areas of disease care with considerable unknowns?

Target-related policy questions:

* What aspects of health care might be/should be/are being targeted for CER?

* Should there be a balance for all of the possible targets selected?

* Must there always be 'cross-target' comparisons (e.g., between treatments and prevention) or should it be type-type targets (drug-drug)?

* How will the various priorities be set and who will do that? If funders of health care fund the studies, is that evidence of conflict of interest? Likewise, if product or service companies fund the studies, is that evidence of conflict of interest?

* Should 'hidden costs,' (e.g., economic and consequences for caregivers) be accounted for in the selection of priorities or are direct medical costs the only interest?

Evidence-type related policy questions:

* Will some types of evidence be preferred over others?

* Should some types of evidence be disallowed?

* Will cross-type comparisons be allowable?

* Since some evidence involves self-report on the part of patients or clinicians, how will accuracy be assured?

Evidence hierarchy-related policy questions:

- * Will there be a preferred hierarchy of evidence?
- * How much evidence is required? What number of studies and quality of studies is required?
- * Must all CER studies account for age, gender, racial, ethnic and health system diversity?
- * Since few studies do account for all those distinctions, what provisions will be made for patients who are in those groups if decisions are made as a result?

Use-related policy questions:

- * How will the results be used?
- * Can results be used to create changes in market entry or payment for products and services?
- * If other agencies (e.g., FDA) have approved products and labels, can CER studies be used to create changes in those regulatory rulings?

Communication-method related policy questions:

- * How will the results be communicated? By whom? In what timeframe?
- * What requirements will there be for updating CER results and for rescinding those published in error, in unclear ways or prematurely?
- * What provisions will be made to account for low health literacy levels and diverse cultural and language groups?
- * Do communicators have a responsibility to monitor errors in 'downstream' communications and misinterpretation of results?
- * Does the communicator of the CER study 'own' the information? For example, if a professional society is selected as the principal communicator of the evidence in their area of clinical expertise, does it 'own' the published information? For areas where more than one professional society serves a field, how would the most appropriate society selection be accomplished?

Implementation- and **outcome-**related policy questions:

- * \$1.1B is hardly sufficient to implement CER. How will adequate funds be secured? Will CER-related user fees be imposed on products and services entering the market? Can private plans or payers supplement funding? Will provider types pay user fees?

* What is the reasonable timeframe for implementing the CER results? How long does a patient, clinician, payer, or health system have to adapt to the change?

* Given contract timeframes, if CER evidence results in recommendations for more costly care (e.g., through increasing volume through improved access, or increasing intensity with higher-cost innovations), what is a reasonable timeframe for adaptation for the health system.

* If new evidence leads to a different conclusion later (as sometimes happens) is the decision-maker (e.g., an AHIP member) held harmless or must they restore payments if the patient has purchased OOP care?

* Will there be oversight of the CER process and if so, who will do that?

* What is the relationship of health care CER to other evidence-based policy making (e.g., The Council for Excellence in Government)?

* How and when will CER effectiveness overall be judged? What metrics will determine its success or failure: process measures such as the number of studies or changes in health care practices, or outcome measures such as lowering of rate of disease incidence or health cost increases?

* If CER becomes a condition of coverage for new drugs, imaging or therapeutic devices, does this unfavorably impact those innovations most needed now? Can exclusions be granted if data are not adequate for comparisons, for rare diseases, or for areas of unmet need?

Comparative Effectiveness and the Patient's Role

The [HHS Federal Coordinating Council for CER research posted its report](#) to Congress and the President on Monday describing federal activities on CER. Another report with actual priority suggestions is due to Congress by the end of July. On Tuesday, the Institute of Medicine released their sage advice about the top 100 priorities as well as a report on CER. I found one line, in particular, of extreme importance from the report to Congress:

“National Institutes of Health (NIH) diabetes prevention trial demonstrated that lifestyle change was superior to metformin and placebo in preventing onset of type 2 diabetes.”

Although an extremely complex and closely watched effort across the private sector, this line is the crux of the issue of success for such an endeavor. Of the entire IOM report, I found the following meaningful:

“Compare the effectiveness of traditional behavioral interventions versus economic incentives in motivating behavior changes (e.g., weight loss, smoking cessation, avoiding alcohol and substance abuse) in children and adults.”

How do we know how much the patient – their genomic and proteomic make-up, their lifestyle choices, their home environment, the food they eat, the exercise they do, the sangria they drank last night, the choices they make, the levers that influence their behavior – impacts our ability to understand the

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health outcomes and economic value of clinical interventions such as medications, procedures or clinical delivery system strategies as proposed by comparative effectiveness research? The fact that CER is now being referred to as patient-centered health research or patient-centered outcomes research is definitely half the battle and we are thrilled to see that, but how do we really put our money where our mouth is to ensure that the patient is, in-fact, informing these efforts and the research knowledge base through both their own actions as well as how they inform interactions with the health care delivery system?

A key finding from the Robert Wood Johnson Foundation's [Project HealthDesign](#) work on personal health records was, "collecting ODL (observations of daily living) data through PHRs gives both clinicians and patients insights that are unattainable in records that capture information only from clinical encounters." How do ODLs play a role in comparative effectiveness research? Well, tracking ODLs can help individuals better understand their own health and well-being status (i.e. pain, mood, sleep patterns, etc.) and/or disease issues. This is, arguably, helpful context to understand who the patient is and how they live their lives and take care of themselves between clinical visits. Put in the right context, individuals can actually visualize trends and relationships in their health data not previously available to them. This alone is helpful, but not likely to impact CER until the information makes its way to a clinician and the health care delivery system. Hence, something about ODL tracking may and should cause individuals to use this information as a "patient" with their clinician. Although this rarely happens today, this phenomenon is on the rise and is playing a critical role with both the clinician in terms of painting a broader picture of the patient sitting in front of them as well as with the patient who is, for the first time in some cases, able to link how they are feeling on multiple levels with a particular experience or clinical indication. This interaction is empowering for both clinician and patient. This interaction has potential repercussions for how the clinician might treat the patient with medical therapies, drugs or other interventions and certainly for how the patient might view their health responsibilities between clinical visits.

So, what does all of this have to do with comparative effectiveness?

Well, if the patient's tracking can influence the provider's clinical decision which influences the comparative effectiveness tracking currently being designed by the federal government, doesn't it make sense to include the patient and enable a technical and operational understanding of their health and health care experiences, decision-making and participation along the way? How can one imagine a clean view of comparative effectiveness without meeting the patient or individual where they are in order to understand how they are potentially influencing comparative effectiveness in their own, personalized way?

Comparative Effectiveness Research from the Health Care Provider Perspective—A Glass Half Full

Why aren't we as health care professionals clamoring for CER? Why shouldn't we encourage health care policy makers and industries to align themselves to how we make clinical decisions? Perhaps we haven't taken the time to fully understand the benefits of CER, how it could directly improve our ability to positively affect the lives of our patients, or we see it as something far-off and impossibly complex to implement. If we take a closer look at CER in relation to our needs as caregivers and front-line professionals attuned to the health needs of our patients, we can positively shape the debate on health care reform through CER.

The benefits of CER to healthcare professionals (HCPs), patients and other stakeholders

Efficacy, established within the confined settings of clinical trials using RCT is a necessary but not sufficient standard for deciding on how to treat patients for their conditions. Meta-analysis alone is not a sufficient way to address the lack of comparative effectiveness information. Performance, in terms of health outcomes, requires HCPs to make our best guess with limited information about which treatments work best for specific patients' conditions.

Instead of settling for "siloes" information on health care treatments, what if we had richer information at our fingertips which evaluate treatments "relative" to one another and for

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specific patient types? By providing that kind of comparative information about treatments, CER gets us closer to the real word of how healthcare professionals practice. Instead of seeing healthcare reform getting between patient and doctor, we might look at CER as a significant step toward healthcare reform providing the necessary conduit between patient and healthcare provider by providing this much needed information to both groups.

Comparative treatment information enables us to choose the best treatments for our patients' conditions. It helps us get to being closer to "right the first time", limiting risk of using the "new" or latest treatments – just because they are new. Medical costs can also be reduced by not having to backtrack and consider more interventions based on failures, or adding on therapies and possibly creating even greater risks or problems, for example drug interactions resulting from unnecessary poly-pharmacy. In addition, disparities could be justified based on the results or outcomes substantiated in CER, rather than just being highlighted for their financial impact.

Patients and HCPs are not the only ones who will benefit from a performance-driven approach enabled by CER. Comparative information will save manufacturers' costs by directing them to develop treatments with the most positive outcomes for the most appropriate patients, and away from me-to products with marginal performance value, directed at the masses. Thus, resources could be directed to developing more data rich and innovative products. Payers will appreciate appropriate costs associated with HCPs prescribing appropriate treatments the first time, resulting in improved patient health and reduced need for treatments or defensive medicine techniques.

CER is not a radical, far-off dream – we can do something now

There are those in the health care field who clearly see and desire the benefits of the performance-driven approach of CER, but are concerned that implementing CER is too daunting in scale and complexity to tackle. Here are three ways to look at addressing these concerns and taking steps towards proceeding with CER:

1- Don't look at CER as an event

Change required for CER is a greater integration of "performance focus" and practices into ongoing organizational operations. Practitioners need to be more engaged with performance and outcomes, and not enticed and drawn to the newest products and technologies until there is reasonable data to support them versus current options. Industry needs to look at innovation from a standpoint of performance versus current options as the litmus test for moving development projects forward, certainly along with other criteria including e.g., patent protection.

2- We've actually been doing this a long time...

Comparative effectiveness is how we, as health care professionals, naturally make health care decisions anyway. A patient performance-driven or outcome-based approach to providing health care is not new. Hospital pharmacists in the late 1970's, with the advent of the Prospective Payment System, began training medical students and residents in teaching hospitals in order to get more appropriate use of medications, by collecting outcomes data through medical records reviews, data based mining from our DRG data bases, and developing guidelines for interventions. In one case, we looked at coronary artery bypass graft (CABG) patients with nutritional depletion prior to surgery to determine whether they did better post op and through recovery based on intervention with total parenteral nutrition (TPN), an expensive treatment in the hospital setting. We were able to prove that the patients did better on this treatment, and in fact the cost of the TPN was offset by the decreased length of stay and other indicators versus the patients who did not receive TPN. The patients on TPN had better outcomes, and had lower hospital charges overall. Needless to say, the study helped to sell a lot of TPN for CABG patients back then. That was CER – in the early days.

3- We're just gaining the support of more stakeholders

CER puts all stakeholders on the same page with common goals. In an attempt to socialize the concept of CER better than it is currently, goals for improving HCP decision-making and patient-performance establishes a frame which we can all understand, agree on, and anchor the discussion. Who doesn't want better health care for themselves, family members or friends? While other goals may be important to CER and health care reform in general, they are often in conflict with other stakeholders' interest, leading to stifled debate and inaction. Why don't we let performance be our lens, and ensure that all other goals align with it? I'd like us to try and socialize CER from the healthcare provider and patient performance perspective.

Successful models already exist for performance oriented approaches. I was a practicing hospital pharmacist when Dr. Roy Vagelos was running Merck. To the healthcare provider on the outside, the company culture operated like a huge medical practice. Patient-performance criteria provided the context for product development and marketing, and the foundation on which business goals and objectives aligned. Fully recognizing those were different times back then – we also need to recognize that the business did well back then too! Why not revisit this successful model in our attempts to socialize and progress the discussion on how to implement CER?

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Disruptive Women in Health Care is a blog dedicated to serving as a platform for provocative ideas, thoughts, and solutions in the health sphere. We recognize that to accomplish this, we need to call on experts outside of the health industry.

The Disruptive Women have audacious hopes for our blog:

- * We're driving change;
- * We're creating chaos;
- * We're finding cures;

...We're disrupting the health care status quo.

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