Comparative Effectiveness Research—Implications for Healthcare

Jeff Goldsmith, PhD

An interview with Jeff Goldsmith, PhD, President, Health Futures, Inc



Jeff Goldsmith, PhD, is President of Health Futures, Inc. and Associate Professor of Public Health Sciences at the University of Virginia. From 1979 to 1990, he was a lecturer in the Graduate School of Business at the University of Chicago, on health services management and policy. From 1982 to 1994, Jeff Goldsmith served as National Advisor for Healthcare for Ernst & Young, and provided strategy consultation to a wide variety of healthcare systems, health plans, supply, and technology firms. Before this, he was Director of Planning and Government Affairs at the University of Chicago Medical Center and Special Assistant to the Dean of the Pritzker School of Medicine. From 1973 to 1975, Jeff Goldsmith worked in the Office of the Governor, State of Illinois, as a fiscal and policy analyst, and Special Assistant to the State Budget Director. He earned his doctorate from the University of Chicago in 1973, studying complex organizations, sociology of the professions, and the politics of developing nations. He graduated from Reed College in 1970, majoring in psychology and classics, earning a Woodrow Wilson Fellowship for graduate study in 1971.

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Correspondence: tcoyote@msn.com **Citation:** *iHealth Connections*, 2011;1(1):36–8

As healthcare costs continue to spiral upwards, healthcare providers and payors have looked to new models to control expenditure. Comparative effectiveness research (CER) and healthcare technology assessments (HTA) have been proposed as important tools to manage escalating healthcare costs. Indeed, in the US, CER is at the center of the current healthcare reform policy. However, while there is strong support for improving the scientific evidence base to inform clinical decision-making, there are concerns that CER/HTAs may have a negative impact on innovation in the pharmaceutical and medical device industries and reduce access to innovation for consumers. As these initiatives are being implemented, there will be a wealth of scientific evidence generated that should reward healthcare innovation. It is also clear that good communication is vital if CER is to be a success. Considerable anxieties need to be overcome to allow the conversion of what has been a craft into a real science in medical practice.

Healthcare costs have been steadily increasing across the globe. In the US alone, healthcare expenditure reached $2.5 trillion in 2009, which translates to 17.6% of the nation's GDP.[1] In 1999, the figure was an estimated $1.3 trillion or 13.8% of GDP. In Westernized nations, an aging population, the growing prevalence of chronic diseases, and the introduction of novel medical technologies (in the form of drugs and devices) has significantly contributed to escalating healthcare costs. In an effort to stem these rising costs, governments, healthcare providers and payors, and healthcare consumers are placing much greater emphasis on value, for new treatments and medical technologies.

In this new era of cost-constraints, comparative effectiveness research (CER), the basic principle of which is comparing the benefits and harms of alternative healthcare technologies or interventions, has been proposed as an important tool to manage escalating healthcare costs. Indeed, CER is at the centre of the current US healthcare reform policy. In 2009, $1.1 billion of federal funds were allocated to support CER, as part of the American Recovery and Reinvestment Act, which is also known as the stimulus bill. The CER funds were divided between the Agency for Healthcare Research and Quality ($300 million), the National Institutes of Health ($400 million) and the Department of Health and Human Services ($400 million). The Institute of Medicine was contracted to determine the priorities for CER in the US.

Variations of CER are already in place in other countries in the form of health technology assessment (HTA) agencies, such as the UK National Institute for Health and Clinical Excellence and the Australian Pharmaceutical Benefits Advisory Committee. These agencies have a common purpose: to demonstrate the effectiveness/usefulness of a procedure or treatment with supportive evidence. The consensus is that CER/HTAs will reduce the knowledge gaps that lead to uneven healthcare provision and provide evidence of value for the current and novel interventions and protocols. However, the counter-argument is that CER/HTAs may be used to control costs by removing choice from consumers.

For leading healthcare industry analyst Dr Jeff Goldsmith, President of Health Futures, Inc. and Associate Professor of Public Health Sciences at

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the University of Virginia, CER represents a mechanism to "inform healthcare coverage and payment decisions going forward, and should deliver reliable information to consumers regarding effectiveness, benefits, and safety related to different treatment options. The research should provide data that compare drugs, medical devices, diagnostics, surgical protocols, and even healthcare delivery—generating evidence that drives CER.

Essentially, CER is envisioned as a key driver of US healthcare reform. Traditionally, from a US perspective, this is an area that has been highly politicized—where lobbying by industry sometimes plays a significant role in decision-making. On the whole, policy-makers and healthcare payors are moving toward an evidence-based framework for making their payment decisions about new and existing technologies—there is a strong belief that money can be saved by emphasizing and supporting technologies that reduce complications or that circumvent the need for more expensive and high-risk therapies. Creating a payment framework to move in this direction cannot be achieved without a strong body of scientific evidence, as well as a professional consensus about what the appropriate solutions to any given patient's problem might be. Generating this knowledge is the goal of CER."

Politics, Policy, and Healthcare

While there is strong support for improving the scientific evidence base to inform clinical decision-making, the concept of CER and HTAs is not without its problems. According to Dr Goldsmith, there are signs of profound resistance to CER within certain sectors: "One issue comes from industry itself—from the pharmaceutical and medical device industries—where the principal focus has been demonstrating safety and efficacy to regulators. This (CER) adds an additional hurdle to surmount, which may negatively affect the magnitude of return on the research investment. This creates a level of uncertainty that could affect the equity value of the company. Additionally, in the US, and possibly many other countries as well, there are suspicions that patients are going to be denied access to new technology, not on the basis that the technology may be effective or ineffective but on the simple basis of cost alone. This will inevitably raise the specter of rationing," Dr Goldsmith said.

These are clearly two significant barriers to CER that not only need to be overcome but also raise considerable political challenges. This means that the adoption of CER in the US is not merely an academic exercise but one that has significant political elements. "Looking at these problems together highlights the extent of the challenge ahead: the imaging and pharmaceutical industries here in the US alone are more than a $500 billion a year business—the size of the GDP of an average-sized country. Certain consumer groups are also very powerful and politically active—organized around advocacy for specific diseases, of which breast cancer is a prominent example", notes Dr Goldsmith. Also relating to this point, much of the discussion to date has highlighted concern that CER will focus on cost rather than value. There is a tremendous hurdle of public education that needs to be surmounted. Fundamental questions that need to be addressed are "how do you get to the point where people consider population health effects as well as the binary effect on an individual's health status?" and "do I get access to the technology or not?" In order for health and finance ministers to be able to use the information provided by CER effectively, consumers need to understand that there will be individual as well as societal benefits.

Communication—Clearing the Path to Success

It is clear that good communication is vital. This was particularly evident during the controversy surrounding breast cancer screening in 2009. A scientific panel appointed by the US Department of Health and Human Services, the US Preventive Services Task Force recommended against routine annual screening mammography in women aged 40–49 years of age.[2] This led to almost violent political reaction in the US—a 'teachable moment', when nobody learned anything. The general public never learned that mammography has a significant false positive rate, 12–15% of tests yield false-positive findings, resulting in adverse health effects on individuals. Such a high false-positive rate not only generates patient anxiety but also leads to a high level of unnecessary and invasive testing, biopsies, etc.

The idea that medical costs need to be considered by individuals, not just finance ministers, and that not all cancers shorten life, or even threaten health in every instance, are lessons that need teaching. These are all issues that should have emerged from the controversy over the mammography screening guidelines; yet they were never discussed because the findings were perceived by the public as a huge conspiracy to deprive women of access to a test that could save their lives. The Department of Health and Human Services panel that evaluated this technology probably did not know how to communicate their findings, which had very significant implications for the health of women and the general public at large.

"It is fair to say that the general public saw this, and other cases like it, as an exercise to deprive them of access, as opposed to improving care. Good communication in the first instance would have helped alleviate the problem. There is tremendous suspicion not only of the motives of government but even of the validity of the scientific information on which government decisions and recommendations are based. I think many members of the general public feel bombarded with conflicting findings about a health practice, and people do not really know how to evaluate those findings with regard to implications for their own health. There is a huge gap between 'public' health and the health of individuals, as they perceive it. Even within the scientific community, there are questions about the reproducibility of research findings, and questions about whether positive findings in a comparative effectiveness study might have been produced by industry or technology advocates looking for a particular answer", says Dr Goldsmith.

"This is a minefield, and a message to the research community must be: unless you can explain in very clear, simple terms to the general public, to patients and their families, how CER is going to be of benefit to them (i.e. by producing safer care, and by avoiding harm) it is going to be very difficult for the CER community to get a fair hearing. In critique of the community that has been doing this research, it appears to have a technocratic bias, that once we 'higher beings' figure out what is good for the general public, they will simply meekly accept our superior wisdom on face value and happily go along with our research."

Comparative Effectiveness Research as an Intersection for Life Sciences and Healthcare

For years, pharmacoeconomic research units in major pharmaceutical companies have been attempting to develop population-level evidence of the benefits of a new drug on the general population or a subpopulation, whether it is older people or people with a particular disease condition. There has been significant industry activity and interest in these issues for years. However, the industry faces a decision about whether to embrace the need for increasing the availability of CER-derived evidence or to use their political power to try and prevent the evidence from being generated in the first place. By definition, CER information belongs to the public, not merely to industry.

Part of the debate also focuses on technologies, drugs, and devices. Furthermore, it is not just about whether these medical technologies add value but also about the importance of balance in terms of CER on healthcare provisions and practices. Essentially, for CER to improve healthcare outcomes, research needs to encompass all aspects of the healthcare delivery system. According to the health reform legislation, CER will include an examination not only of medical interventions but also medical procedures, preventative programs, and diagnostic tests.

The enabling legislation for the Patient Centered Outcomes Research Institute (PCORI), which was created by the Patient Protection and Affordable Care Act,[3] was very broad. PCORI's mission is to support the production of well-validated scientific evidence to assist in the making of informed decisions about a broad range of healthcare-related issues. "It did not merely cover drugs and devices, but procedures, diagnostic tools, integrated health practices, healthcare interventions, protocols for treatment, care management, and, an astonishingly broad category, 'other strategies using treatment management or diagnosis in prevention of illness or injury'. This legislation covered literally everything that we do in the healthcare system, not just the tools that we use to do the job with", notes Dr Goldsmith.

The Role of Healthcare Data

Healthcare data are being used by the industry in terms of pharmacoeconomic analysis, and also for pharmacovigilance—safety. Significant to this, and to the increased digitization of healthcare data, was the passage of the economic stimulus bill in early 2009. The stimulus bill focused on the physician adoption of electronic health records (EHRs), with the belief that the closer one got to the patient, and to the physician's prescribing and ordering decisions, the more one would learn about the subsequent course of a patient's illness and the effect of the treatment pathway the physician directed. "This cannot be achieved with traditional hospital records alone. Many people believe that the investment in physician adoption of EHRs, which has lagged in the US, was a vital investment if the effect of a particular decision to use or not use a technology is to be tracked. To map the health status of patients down the line, and to provide this incredibly granular database, patients need to be monitored through time. Further investments, however, are still needed to continue to improve the volume and quality of CER," says Dr Goldsmith.

With the growing amount of data, care must be taken to ensure that these data are used intelligently. With the increasing digitization of patient information, when information is being stored in relational databases, it should be a prerequisite that these powerful databases are used in 'thoughtful' ways. However, the secondary analysis of these data, to analyze the trajectory of patients that have had a particular course of treatment, comparing it to the trajectory of patients with the same type of illness and intensity of illness, did not fulfill this prerequisite. This all points to the need for more powerful tools to better analyze the effect of a given treatment or set of treatment decisions on patient outcomes.

As these initiatives are being implemented, in terms of the studies themselves and understanding how to perform the studies to produce good evidence, there will be a wealth of scientific evidence generated through CER that should reward healthcare innovation. According to Dr Goldsmith, "this enables the idea of value-based insurance design, where exceptionally effective technologies carry lower rates of patient cost-sharing. Perhaps, in some instances, the insurer might actually offer positive financial incentives to promote use. In other words, not cost-sharing but actually paying 'customers' to use the technologies because there is such a huge return on health regarding a particular condition. So, certainly within the US, more of this will focus on trying to implement financial incentives to foster the adoption and use of technologies that generate an exceptional return, both to individuals' and society's health. There is also a belief on the part of the manufacturers that, if they can demonstrate a significant return to society for the use of their technology, they have a legitimate claim to higher payment rates than comparable technologies that exist in the market place. There is also an argument that convincing evidence of comparative effectiveness may lead to more-favorable decisions about coverage and payment—leading to the debate of payments being based on value, rather than mere availability."

Concluding Remarks

As CER takes hold, a potential vision of healthcare provision in 20 years time becomes apparent. It will be commonplace to use electronic tools to manage the care process to help physicians and patients alike—making evidence-based intelligent decisions right at the point-of-care. In other words, the output of CER helps to create a 'global positioning system' for clinicians to use in patient care decision-making, locating the patient and where they are 'right now' in their degree of risk, and using evidence-based decision support to get the patients to a 'healthier place'—this is the Holy Grail. CER is here to stay so the question is: how can we surmount the considerable anxieties over converting what has been a craft into a real science in medical practice? ■

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