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What is Comparative Effectiveness Research, and Why is it Being Badmouthed?

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For all the talk of U.S. being at the forefront of medical research, we do not know if many of the tools we use in health care — drugs, devices, screening and treatment protocols — actually work and therefore can not calculate whether their benefits outweigh their risks. Nor do we know if they constitute the best treatment for specific patients, or under particular conditions. Collectively, much of the care we get is duplicative, ineffectual or harmful. At the same time, many tried-and-tested preventive and therapeutic strategies are woefully underused. Indeed, in a study published in 2003, researchers looking at a sample of U.S. adults found that Americans receive appropriate care only 55 percent of the time.

More recently, concern over health care costs has led many scholars to question whether or not we are getting a good return on our investment. The question is particularly pertinent because the U.S. ranks #1 — overall and per capita — in what it spends on health care among developed nations, but our health care system leaves many people out and lags in terms of favorable health outcomes.

Given the need to enhance value in our health care systems, many experts and policy-makers support the creation of a center for comparative effectiveness research (CER) in the U.S. The center would study the effectiveness of different treatments for the same illness, gathering solid evidence to document what works for whom, and at what cost. At present, Britain, France, Australia and Germany are at the forefront of this type of research, using their findings to make decisions concerning what treatments to promote and what services to cover.

The American Recovery and Reinvestment Act of 2009 allocated $1.1 billion to support CER within the U.S. Department of Health and Human Services and created a Federal Council to coordinate the CER efforts. The goal is to increase the knowledge base to help health care providers decide what treatments are the most likely to benefit their patients. But because some opponents of the idea have visions of apparatchiks with checklists hovering over practitioners or denying payment for care, the legislation precludes the Council itself from enacting clinical guidelines and from making coverage and reimbursement decisions.

CER is not altogether new to the U.S. Between 1972 and 1995 the U.S. Congress had an Office of Technology Assessment whose mission was to provide objective analysis of the scientific and technical issues of the late 20th century, including the beneficial and adverse effects of emerging technology. Although the agency's scope went beyond health and health care, it provided evidence on a variety of devices and treatments, from AIDS prevention to wheelchairs. Since the agency's demise, however, this task has been only carried out only partially and sporadically.

At present, the Agency for Healthcare Research and Quality (AHRQ) has a limited mandate to conduct research on the effectiveness and appropriateness of various health services, including prescription drugs. The agency contracts with 13 Evidence-based Practice Centers affiliated with universities and private organizations to synthesize existing knowledge. The agency also generates new research by working with other health care organizations that have large databases at their command. Current efforts in CER have also been supplemented by private sector efforts. For example, Blue Cross and Blue Shield operate a Technology Evaluation Center that reviews existing clinical evidence to assess the appropriateness and efficacy of certain procedures, drugs, or devices. Similarly, other health plans also evaluate evidence to provide a basis for their drug formularies and the devices and procedures they cover.

The need and rationale for CER has prompted broad-based interest in systematizing and expanding this effort, making sure that the knowledge gained reaches practitioners and is available to patients. This would ensure that more health care decisions are based on evidence rather than hunches, and that ineffective, potentially harmful, costly treatments are avoided. CER has therefore been endorsed by the Institute of Medicine, the Medicare Payment Advisory Commission, America's Health Insurance Plans, and the American Medical Association, among others.

But at the same time, CER has earned the wrath of some who want to perpetrate the idea that the comparative effectiveness research is the 'entering wedge' for government to dictate medical practice and decide who gets what service. CER has therefore become a boogeyman. Opponents have used scare tactics against it, brandishing a number of allegations concerning what it is and what it will do. Let us examine some of these:

Myth #1: *CER will insert government into the patient-doctor relationship.* This was the same argument that was used to delay or obstruct third-party payments by insurers many years ago. Then, it assumed that it was the exchange of money for care that defined patient interaction. Now, the argument is equally specious. Having more evidence of what works for whom in no way interferes with the relationship between physician and patient. Indeed, it can promote a more egalitarian relationship, facilitate communication on the risks and benefits of different treatment options and, most importantly, improve patient outcomes.

Myth #2: *CER is driven primarily by a desire for cost control.* While it may very well be — and should be — that CER will lead to the discontinuation of practices that are not effective and can even be harmful, there is no assurance that this will save money. Indeed, because CER can also identify beneficial treatments that are now underused, it may result in certain services being made available more widely or more intensively. Moreover, there is no assurance that, in comparing two very different types of treatment, the least expensive one will prove to be the more effective one. CER is aimed primarily at providing evidence, giving decision-makers — both providers and patients — a firmer ground on which to base their decisions. Any savings resulting from this will be strictly a bonus.

Myth #3: *CER will restrict choice.* Much of the current gamut of choices involves practices of dubious value. In addition, "choice" often entails a process of trial-and-error that is costly in terms of both suffering and money. CER will broaden some options for care and restrict others. Its aim, however, is to build evidence that will allow physicians and other health providers to identify which treatments are most likely to benefit their patients. As ethicists

Ruth Faden and Jonathan Moreno have succinctly expressed it, "an uninformed choice is like no choice at all."

Myth #4: *CER is a way to ration care.* This argument plays on people's fears that some treatments may not be available as a result of CER. That may indeed be the case if there is evidence that some treatments serve no useful purpose, are inferior to other options, or are downright harmful. But the deliberate separation of the proposed CER Council from clinical guidelines and reimbursement decisions protects against this possibility.

Additionally, this argument assumes that care is not being rationed at present, which is not the case. Services are rationed by price: if you cannot afford to pay for a given service or your insurance does not cover it, you face definite barriers to care.

Myth #5: *CER will focus on majority populations, neglecting the fact that treatments can affect different groups in different ways.* Another way of expressing the same "rationing" idea is the statement that "CER promotes 'one-size-fits-all' health care." Some groups are therefore worried that effectiveness will be judged on average results across the entire population rather than on effects on particular individuals. This is something that is already occurring. As Alan M. Garber and Sean R. Tunis have recently stated, "…with too few appropriately designed studies, physicians, patients, and families have often had little guidance about which patients were most likely to benefit from a clinical strategy." This concern can be addressed in the design of the research strategies employed to assess different treatments. The 'comparative' part of the research should therefore refer not only to different treatments but also to a variety of populations, which should be included in sufficient numbers to warrant reliable conclusions.

As with most other health-related research, the findings of comparative effectiveness research will be contested, argued about, and fought over. CER, like most policy issues, will have winners and losers. It is therefore not surprising that that the topic has been described as a "medical minefield." But, as ethicists Faden and Moreno point out, "Choosing blindly is an empty right; choosing with evidence respects patients' rights and enhances quality. This is a case in which good ethics demands good facts."