

AAFA POLICY MEMO

COMPARATIVE EFFECTIVENESS RESEARCH

As America pushes to improve quality and reduce costs, leaders want more evidence that recommended treatments actually work better than others do. Comparative Effectiveness Research (CER) can be an important tool, yet any legislation promoting CER must contain safeguards to protect patient from unintended outcomes that do not support optimal patient care. This paper explores the meaning and implications of CER and recommends a policy course for asthma and allergy advocates.

The current push toward reforming America's health care has generated discussion around the idea of designing and conducting research to assure better treatments and patient care while reducing costs. Yet, experts and advocacy groups alike have challenged the potential benefits of such research. This policy memo aims to address important questions about CER.

1. What is Comparative Effectiveness Research? How does this differ from Evidence Based Medicine?

CER and EBM are both related yet distinct. Dr. David Sackett defines *evidence-based medicine* (EBM) as "the conscientious, explicit, and judicious use of current best evidence in making decisions about the care of individual patients." The purpose of EBM is to improve the amount as well as increase the quality of health care delivery and health outcomes. EBM emphasizes the need to integrate medical literature and patient preferences into the standard of care when practicing medicine.

In recent years, as the health industry has searched for ways to contain costs, it has placed increased emphasis on the comparative value of health interventions. Thus, comparative *effective research* (CER) is the most recent iteration of designing evidence development to improve and inform medical decision-making. CER is viewed as a critical puzzle piece to expanding the evidence landscape and successfully implementing EBM.

2. How did CER emerge as a key strategy for containing health costs?

Congress began considering EBM and CER as early as 1972, underscoring the urgency of assessing the value of new medical technologies, particularly those that were quickly adopted into the health care market. By the late 1980s and early 1990s, these efforts had lost steam.

A later effort by the Blue Cross Blue Shield Association (*RxIntelligence*), which was intended to conduct cost-benefit and cost effectiveness studies on pharmaceutical drugs and provide evaluation of drugs, failed after two years. These failures occurred because of a lack of federal-level support as well as effective opposing stakeholders, such as the medical products industry.

A report finding shocking variability of medical practice across the nation helped to resuscitate the issue in the 1990's. This momentum led to the creation of the federal Agency for Healthcare Research and Quality (AHRQ) to support research on the

outcomes of health care services and procedures, including how to reduce costs and broaden access to essential services.

Momentum has continued to build to expand the role of EBM and CER. For example, in 2006, a former administrator of Medicare, Gail Wilensky, published a high profile proposal for a multi-billion dollar center that could create useful information for both payers and patients and improve decision making. The following year, the Blue Cross and Blue Shield Association (BCBSA) proposed legislation to establish a new, independent institute funded by all health care payers to conduct research comparing the effectiveness of new and existing medical procedures, drugs, devices and biologics.

The issue has had a global impact. For example, the United Kingdom's National Institute for Health and Clinical Excellence (NICE) reviews all types of health technologies and drugs that may have a significant health or fiscal impact on the Britain government-run health plan, the National Health Service (NHS), and guides NHS on the use of new and existing technologies.

How Does Congress Propose to Support CER?

For the past two years, Congress has been considering legislation to oversee and fund CER activities by the federal government. The Stimulus Bill (ARRA) contained funding for CER through 2010 and enabled a regulatory oversight body under the Department of Health and Human Services. However, Congress is likely to propose a more permanent structure and funding stream.

Two of the major health care reform bills before the Congress include the CER provisions that would expand the role of the Agency on Healthcare Research and Quality for CER. Two other bills would establish a public-private partnership for CER with oversight by a not-for-profit non-governmental entity.

Who is Influencing the Push for CER Legislation, and What Do They Want?

The biggest current push is coming from parts of the federal government. The House and Senate are both expected to propose include CER in any health reform bill considered this summer. Congressional Democrats, who lead Congress, hope for support from Republicans. The Obama administration has emphasized the need for containing health care costs, and Peter Orszag, Director of the Congressional Budget Office has said that health reform legislation must be budget neutral. Yet, he adds that CER will not be used for rationing health care. Some Congressional Republicans have used the prospect of health care rationing to attack CER and health care reform. Senator Jim Kyl has introduced a bill prohibiting Medicare or Medicaid from using CER to deny coverage. Republicans leaders are expected to use this message about CER to lead into the specter of socialized medicine.

The health care industry is active on this issue. In general, while health insurers are major proponents of CER as a tool to inform coverage issues and reduce medical costs, while providers and pharmaceutical manufacturers are skeptical about creating a structure that interferes with medical decision-making and reduces revenues from drugs that are deemed no more effective than cheaper alternatives.

Patients and consumers have also organized. CER is one of the leading issues for the **National Health Council (NHC)** (AAFA is a member). NHC has adopted principles on CER and is promoting them to members of Congress. NHC believes that patients should have a meaningful voice in any CER oversight body, that research should focus on both treatment and delivery options and that CER must not determine coverage or reimbursement policies until such conclusions are tested in real world settings as they impact individuals and various subpopulations.

Additionally, the **National Working Group on Evidence Based Healthcare** (AAFA is a founding member and the only asthma/allergy focused member) was convened by Mental Health America in 2006. Its core goals include support for individualized care and not “one-size-fits-all” treatment; that acceptable research evidence should not be limited to randomized clinical studies; that all types of research should be incorporated to ensure all racial/ethnic groups are included; and that safety and finding the right treatment for the individual should be a top goal of evidence-based healthcare. Its activities have focused on promoting patient advocacy at the state and local level by convening regional forums and developing advocacy materials. The Working Group has supported legislation to create a national, independent CER body that includes patient representation, as championed by Senator Max Baucus.

How does AAFA Approach CER?

CER may offer tremendous benefits to patients as relevant research emerges that will help guide them in the care and prevention of illness. Physicians will have better information to guide their recommendations. However, CER can have limitations for asthma and allergy patients if individual needs could get lost in conclusions about what works best for the majority of patients. Children and certain ethnic minorities experience a disproportionate burden of asthma. Yet, historically, much of the clinical research in this nation had white males as subjects. Even now, as women and minorities are actively recruited as research subjects, they remain underrepresented in clinical trials. As another area of concern, clinical trials are limited to healthy subjects and may not include people with asthma or allergies. These outcomes may have limited value for those who have one or more chronic diseases. Further, individual genetic makeup has an impact that cannot be predicted with certainty by comparative studies on a general population.

As we cannot ignore the potential benefits and limitations of CER, we cannot ignore the inevitability of CER, whether performed in the private or the public sector.

AAFA will work in concert with our primary coalition partners to shape a response to legislative proposals that honors our principles and advocate alongside other patient groups. AAFA will encourage –

- **An important role for patients.** Patients should be included as voting members of the governing board of any new entity to oversee comparative effectiveness research activities.
- **Broadly focused CER** not limited to randomized clinical trials. CER must take into account the real world situations of patients. For instance, asthma patients who smoke typically are not research participants. However, many adult asthma patients

have smoked and may react differently to medicines than those who have never inhaled tobacco smoke directly into their lungs.

- **Transparency of evidence based decision-making** to help assure that CER is not used inappropriately to drive coverage decisions by Medicare, Medicaid, and other government or private payers.

AAFA will review and assess proposals against the principles articulated by the National Health Council and the National Working Group for Evidence Based Medicine. Based on these principles, AAFA will engage in appropriate advocacy activities including contacting members of Congress, and encouraging others to do so using AAFA's communication resources.