

in scope, the recommendations may be more influential than they might otherwise have been, but the report is unlikely to quell the controversy surrounding CER.

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Mr. Iglehart is a national correspondent for the *Journal*.

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## Comparative-Effectiveness Research — Implications of the Federal Coordinating Council's Report

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Despite a plethora of diagnostic and treatment options, practical information that can guide health care choices for an individual patient are often elusive, and the resultant clinical uncertainty is an important factor driving regional variations in clinical practice. Clinicians and patients need to know not only that a treatment works on average but also which interventions work best for specific types of patients. Comparative patient-centered information is essential to translating new discoveries into better health outcomes, accelerating the application of beneficial innovations, and delivering the right treatment to the right patient at the right time.<sup>1</sup>

The American Recovery and Reinvestment Act (ARRA) provided support for comparative-effectiveness research (CER), which has recently been referred to as “patient-centered outcomes research.”<sup>2</sup> The purpose of CER is to provide information that helps clinicians and patients choose the options that best fit the individual patient's needs and preferences. CER is already conducted by the Agency for Healthcare Re-

search and Quality (AHRQ), the National Institutes of Health (NIH), the Department of Veterans Affairs (VA), and others, but the ARRA substantially increased the federal investment in CER, providing \$400 million for the Office of the Secretary in the Department of Health and Human Services (DHHS), \$400 million to the NIH, and \$300 million to the AHRQ. It also established the Federal Coordinating Council for Comparative Effectiveness Research to foster optimal coordination of CER conducted or supported by the federal government. On June 30, the Council released a report to President Barack Obama and the Congress on its recommendations for CER funding priorities for the Office of the Secretary.<sup>3</sup> This report, along with one from the Institute of Medicine (described by Iglehart on pages 325–328), will inform the operational plan of the secretary of health and human services for \$1.1 billion in CER funds. We serve as the Council's executive director and the director of AHRQ, but the report reflects public input and contributions of all Council members and many others.

The Council's vision is to lay the foundation and build the infrastructure for CER to develop and prosper so it can inform decisions made by patients and clinicians. The Council specifically identified high-priority research gaps and one-time investments in infrastructure that would accelerate the conduct of CER by multiple researchers. We set three main objectives: to develop a definition, establish prioritization criteria, create a strategic framework, and identify priorities for CER; to foster optimal coordination of CER conducted or supported by federal departments; and to formulate recommendations for investing the \$400 million provided to the Office of the Secretary.

To establish a transparent, collaborative process for making recommendations, the Council sought public input through three public listening sessions and extensive commenting on its public Web site. The Council heard from hundreds of diverse stakeholders and received feedback on draft documents.

We defined CER as the conduct and synthesis of research comparing the benefits and harms

### Threshold and Prioritization Criteria Outlined by the Federal Coordinating Council for Comparative Effectiveness Research.\*

#### Minimum threshold criteria for projects (must be met for a project to be considered)

- Inclusion within statutory limits of ARRA and the Council's definition of CER
- Potential to inform decision making by patients, clinicians, or other stakeholders
- Responsiveness to expressed needs of patients, clinicians, or other stakeholders
- Feasibility of research topic

#### Prioritization criteria for scientifically meritorious research and investments

- Potential impact (e.g., prevalence of condition, burden of disease, variability among outcomes, costs)
- Potential for evaluating comparative effectiveness among diverse populations and engaging communities in research
- Addressing of uncertainty within the clinical and public health communities regarding management decisions and variability in practice
- Addressing of a need or gap unlikely to be addressed through other organizations
- Potential for multiplicative effect (e.g., laying of a foundation for future CER, such as data infrastructure and methods development and training, or generating of additional investment outside government)

\* ARRA denotes the American Recovery and Reinvestment Act, and CER comparative-effectiveness research.

of various interventions and strategies for preventing, diagnosing, treating, and monitoring health conditions in real-world settings. The purpose of this research is to improve health outcomes by developing and disseminating evidence-based information to patients, clinicians, and other decision makers about which interventions are most effective for which patients under specific circumstances. The Council established explicit threshold and prioritization criteria to guide recommendations for funding priorities (see table). The Council also developed a strategic framework for categorizing current CER activity, identifying gaps, and informing our recommendations for priorities. The framework supports immediate decisions and provides the foundation for longer-term strategic decisions on CER priorities and related infrastructure.

CER investments and activities can be grouped into four major categories: research, human and

scientific capital (e.g., training of new researchers or development of methods), data infrastructure (e.g., distributed data networks, registries, or linked longitudinal administrative data), and dissemination and translation into practice. Investments in cross-cutting "themes," including high-priority populations, conditions, or types of interventions, could span more than one category of activity, and investments should be leveraged for additional uses (e.g., data-infrastructure work that also supports research on high-priority populations).

In making recommendations, the Council aimed to respond to the needs of patients and clinicians, balance the achievement of near-term results with the building of longer-term opportunities, and capture the unique role that the ARRA funds could play in filling gaps and building the foundation for future CER. The Council recommended that the primary area of investment

for this funding be data infrastructure, which could include projects such as the linking of current data sources to enable researchers to answer comparative-effectiveness questions or the development of distributed electronic-data networks, patient registries, or partnerships with the private sector.

Recommendations for secondary investments include the dissemination and translation of CER findings and investment in cross-cutting projects focused on high-priority populations or interventions. The specific populations identified by the Council were racial and ethnic minorities, persons with disabilities, persons with multiple chronic conditions (including coexisting mental illness), the elderly, and children. CER will be an important tool for informing decisions that affect these populations and reducing health disparities. High-priority interventions include medical and assistive devices, procedures or surgery, behavioral changes, prevention, and delivery systems. For example, behavioral changes and prevention have the potential to decrease the rates of obesity and smoking and boost adherence to medical therapies. Delivery-system research, such as studies comparing various processes for hospital discharge or differing community-based care models or studies testing the health effects of various medical-home models, have substantial potential to drive better health outcomes.

The Office of the Secretary's funds may also play a supporting role in research and human and scientific capital. Because the Council anticipates that the AHRQ, the NIH, and the VA will contin-

ue to play major roles in these essential CER activities, the Secretary's funding would probably focus on gaps in their portfolios.

The expansion of CER, or patient-centered outcomes research, has at least three major implications. First, the results of such research will better inform a broad array of health care decisions. Second, the ARRA's provision for CER represents a significant investment in one of the translational steps toward improving the quality and value of health care for all.<sup>4</sup> Health services research, of which CER is only a part, has been estimated to account for 1.5% of total biomedical research expenditures and 0.1% of total U.S. expenditures on health care,<sup>5</sup> but the ARRA funding may reflect a trend toward increased investment in these translational building blocks for improving health. This investment creates the potential for training a new cadre of researchers, invigorating current researchers, and improving health outcomes.

Third, CER has the potential to drive high-value innovation and to enable the practice of more personalized medicine based on subgroups of patients. The goal of randomized efficacy trials is often to prove that a treatment

is superior to placebo. But more important questions may be whether the intervention is better than other available interventions for specific populations and whether we can identify the subgroups of patients who will benefit the most from (or are the most likely to be harmed by) specific interventions. CER must focus on informing the care of people who are often excluded from trials (e.g., those with multiple chronic conditions) and identifying subgroups of patients (e.g., the elderly, racial and ethnic minorities, or people with a particular genetic marker) whose response to a given therapy or intervention may be different from that of the "average" patient in a trial.

This unique opportunity to invest in a major component of the scientific infrastructure for improving health care delivery will be indispensable for achieving a health care system that delivers affordable, high-quality care for all Americans. Physicians and patients deserve the best patient-centered evidence regarding what works, so that Americans can receive care of the highest quality and the best possible outcomes can be achieved.

The views expressed in this article are those of the authors and do not represent official policy of the DHHS or the AHRQ.

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