AHRQ Update

Commentary: A Progress Report on AHRQ's Effective Health Care Program

Carolyn M. Clancy, and Jean R. Slutsky

In previous issues of this journal we have described the authorization and structure of Agency for Healthcare Research and Quality (AHRQ's) Effective Health Care Program (Clancy, Slutsky, and Patton 2004). In this issue, we would like to update you on its progress, describe the interest that the Effective Health Care Program has garnered recently, and discuss some observations that we have made since the program began.

As you well know, this is a very interesting time in the history of health and medicine. Our investments in biomedical research have resulted in many new diagnostic and therapeutic options. Through the efforts of the readers of this journal, cutting-edge health services research is yielding innovations that are changing how we practice medicine and make policy.

Clinicians and patients now have many choices for treating common illnesses such as hypertension, heart failure, HIV, mental illness, and other chronic illnesses, and unprecedented innovations in diagnosis and prediction bring us closer than ever to a vision of personalized health care. We also are beginning to see how information technology can bring information immediately to clinicians, patients, and others when and where they need it. Health information technology (IT) greatly expands our ability to diffuse information quickly and efficiently throughout the health care system. Health IT also is enhancing our capacity for research as a natural by-product of delivering health care.

Along with realizing new potentials and opportunities, we have learned that new options bring new challenges both in how we assess the safety and effectiveness of different therapeutic choices and who would benefit most from their use.

One of the first of these challenges is how to evaluate these innovations and determine which represent added value, which offer minimal enhancements to current choices, which fail to reach their potential, and which work for some patients and not for others. The need to develop better evidence about the benefits and risks of alternative choices is imperative.

The mission of AHRQ is to improve the quality, safety, efficiency, and effectiveness of health care for all Americans. Effectiveness sits squarely in our mission—what is the right treatment for the right patient at the right time.

Comparative effectiveness research is a means to an end. Our mission is fulfilled when health care decision makers—including patients, clinicians, purchasers, and policymakers—use up-to-date, evidence-based information about their treatment options to make informed health care decisions. The key to success for this research is that it provides evidence that informs the choices confronting clinicians and patients and, where possible, should closely align with the sequence of decisions they face.

As directed by Section 1013 of the Medicare Modernization Act, (Public Law 108–173) which authorized the Effective Health Care Program, AHRQ also needs to ensure that findings are frequently revisited, so they remain relevant and up-to-date. New evidence and findings will become part of the cycle of keeping comparative effectiveness findings current and relevant through regular updates.

PROGRESS AND IMPACT

To date, AHRQ has released eight comparative effectiveness reviews. These reviews can be found on the Effective Health Care Program website (<http://effectivehealthcare.ahrq.gov>), discussed above. They are:

Gastroesophageal Reflux Disease (GERD)

For management of gastroesophageal reflux disease, medications called proton pump inhibitors can be as effective as surgery in relieving the symptoms and improving quality of life.

Breast Cancer Diagnosis

Among women who receive an abnormal mammography findings or physical exams, four common noninvasive tests (magnetic resonance imaging, ultrasonography, positron emission tomography scanning, and scintimammography) are not accurate enough to routinely replace biopsies.

Managing Anemia In Cancer Patients

Among cancer patients undergoing chemotherapy or radiation, there is no clinically significant difference between epoetin and darbepoetin in the management of anemia. The drugs show no clinically significant difference in improving hemoglobin concentration and reducing the need for transfusion.

Osteoarthritis Drugs

Nonsteroidal antiinflammatory drugs (NSAIDs) and COX-2 inhibitors present similar increased risks of heart attacks while offering about the same

level of pain relief for patients with osteoarthritis. The exception is naproxen, which presents a lower risk of heart attack for some patients than other NSAIDs or COX-2 inhibitors.

Renal Artery Stenosis

Increasing numbers of patients with narrowed kidney arteries are undergoing vessel-widening angioplasty and placement of a tubular stent, but evidence does not show a clear advantage of that treatment over prescription drug therapy.

Off-Label Use Of Atypical Antipsychotics

Some newer antipsychotic medications approved to treat schizophrenia and bipolar disorder are being prescribed for depression, dementia, and other psychiatric disorders without strong evidence that such off-label uses are effective. Research is urgently needed for new treatments of dementia patients with severe agitation.

Second-Generation Antidepressants

Today's most commonly prescribed antidepressants are similarly effective to first-generation antidepressants and provide relief to about six in 10 patients, but current evidence is insufficient for clinicians to predict which medications will work best for individual patients. Six in 10 patients experience at least one side effect, ranging from nausea to sexual dysfunction.

Oral Diabetes Drugs

Most oral medications prescribed for type 2 diabetes are similarly effective for reducing blood glucose, but the drug metformin is less likely to cause weight gain and may be more likely than other treatments to decrease so-called bad cholesterol.

In January 2007, AHRQ released the first summary guide for consumers and clinicians derived from a comparative effectiveness report. The consumer report, titled *Choosing Pain Medicine For Osteoarthritis*, translates the information from the comparative effectiveness report on osteoarthritis drugs into language that will help consumers choose among their treatment options. The companion guide, *Choosing Nonopioid Analgesics for Osteoarthritis*, further synthesizes the evidence into a resource that can help clinicians work with their patients to make informed decisions about treatments for osteoarthritis.

In addition to comparative effectiveness reviews, the Effective Health Care Program has supported methodological research that will help advance the quality of comparative effectiveness research. In May 2007, AHRQ published *Registries for Evaluating Patient Outcomes: A User's Guide*. This document had over 30 authors and uses numerous case studies to illustrate the report's findings.

AHRQ has a series of upcoming reports that deal with critically important issues facing the health care system. They include:

- Angiotensin-converting enzyme Inhibitors (ACEIs) versus angiotensin II receptor antagonists (ARBs) for high blood pressure.
- Surgery versus stents coronary artery disease.
- Medications and other treatments (e.g., diet, exercise) for low bone density.

Although the program is only two years old, we are beginning to see what impact it can have. For example, Consumer Reports Best Buy Drugs, a public education project of Consumers Union, uses findings from Effective Health Care Program to help clinicians and patients determine which drugs and other medical treatments work best for certain health conditions.

Best Buy Drugs contracts with teams of physicians and researchers who translate the technical reports into layman's terms. The information is finalized into three final consumer products: a full report, a three-page summary, and a booklet that can be downloaded at no charge (www.crbestbuydrugs.org).

The magnitude of the EHC program's impact is evidenced by the fact that the Consumers Union drug class reviews are downloaded at a rate of 110,000 per month. Over the course of the 2-year project, over 1 million reports have been downloaded. In addition to disseminating the consumer materials and reports via the website, Best Buy Drugs has an outreach program that links to existing groups with statewide reach and credibility throughout the medical community.

The National Business Group on Health uses findings from the Effective Health Care Program in its evidence-based benefit design program. Medscape offers CME based on comparative effectiveness reviews and numerous other organizations that use the findings in their deliberations on patient care, formulary design, and areas for needed research.

NEW INTEREST, NEW PROPOSALS

On June 12, 2007, I had the privilege to testify before the House Ways & Means Health Subcommittee about the issue of comparative effectiveness (Statement on AHRQ's Research Efforts in Comparative Effectiveness 2007). The hearing came on the heels of a series of proposals that have been floated to broaden the funding for and the scope of effectiveness research. It is very thrilling to see the growing interest in, and attention to, enhancing the role of comparative effectiveness research in our health care system.

I was very pleased and proud that all the witnesses—and members of the subcommittee—were extremely complimentary of AHRQ’s long track record in effectiveness research and now comparative effectiveness research through our Effective Health Care Program. It is a testament to the hard work of AHRQ’s staff and researchers that this Agency and its work are so well received.

One of the witnesses, Representative Thomas Allen (D-ME) described the comparative effectiveness legislation that he cosponsored Representative Jo Ann Emerson (R-MO). The legislation, H.R. 2184, The Enhanced Health Care Value for All Act of 2007 (H.R. 2148), authorizes \$3 billion for research by AHRQ. Recognizing that better clinical information on health care products and services is a public good, the legislation sets up a public–private funding structure, which will receive federal funding as well as funding from health insurance plans and large employers with self-insured plans. On August 1, 2007, the U.S. House of Representatives passed a legislation that included the Allen/Emerson proposal.

The Act also would establish a Comparative Effectiveness Advisory Board appointed by the Comptroller General of the United States. The board will include private health care payers including employers and consumers, health care providers, health care researchers, health care consumer representatives, and health industry representatives.

Another witness, Gail Wilensky, Ph.D., Senior Fellow at Project Hope, described the proposal that she first outlined in the November/December 2006 issue of *Health Affairs* (Wilensky 2006). She describes four potential options for the creation and placement of a new comparative effectiveness center.

The role of the Center for Comparative Clinical Effectiveness would be to help inform clinical decisionmaking and provide guidance on reimbursement and new requirements for coverage. These are fundamental and critical differences in roles.

- Option 1: Placing the center within AHRQ. The new center would also have an independent external board, along with a panel of experts to advise on research priorities and to provide oversight for the monitoring of research contracts and the dissemination of results.
- Option 2: Placing the center and board elsewhere within the Department of Health and Human Services.
- Option 3: Placing the new center within a quasi-governmental entity such as the Institute of Medicine, Federally Funded Research and Development Centers, and public foundations.

- Option 4: Placing the center within the private sector. This could be in a freestanding institution or one affiliated with a university or other entity.

A third proposal to support comparative effectiveness research (Serota) has been floated by the Blue Cross Blue Shield Association would have Congress pass legislation creating a new institute that would support research comparing the effectiveness of new and existing procedures, drugs, devices, and biologics based on four key principles:

- Funding should be ensured by asking all payers—government and private—to contribute;
- The Institute should support a broad range of research, especially clinical trials;
- Significant education programs and incentives are needed; and
- The new Institute should be governed by a board with both public and private representation.

The proposal recommends an initial budget of approximately \$500 million annually.

Health IT

I would like to mention briefly the role of health IT, which will make it easier for researchers to gather information for their research and for users of research findings to get information in real time when they need it. The health care system's growing investments in health IT provide us with an unprecedented opportunity for redefining the possibilities of observational studies, accelerating and targeting the uptake of relevant information, and providing feedback to the biomedical enterprise itself.

Health IT will make it possible for research to answer the pressing questions facing the health care system more quickly and efficiently. In the future, health IT will provide us with the vehicle for transforming our health services research enterprise so that we can evaluate the effectiveness of interventions and treatments in real time as a byproduct of providing care.

AHRQ's Fiscal Year 2008 budget request includes \$15 million for a health care initiative that will begin the infrastructure for a federated system of databases that can help answer critical comparative effectiveness questions (Budget Estimates for Appropriations Committees 2007). This system would enable researchers to match treatments and outcomes, and in that way learn

from the nation's day-to-day medical practice and improve safety and effectiveness of medical treatments. This initiative is not meant to replace clinical trials but to allow us to do research in ways that we cannot use randomized clinical trials effectively—to answer questions of external validity, impacts on subpopulations, effects of complex medical situations, and rare events.

Health IT also will greatly improve the ability to diffuse evidence and information more quickly throughout the health care system. For example, clinical decision support tools will make it possible to deliver relevant information to clinicians and patients, at the point of decision making. Most commonly envisioned as a pop-up reminder on a screen, clinical decision support should include information communicated directly to patients and caregivers at home—by phone, computer, or by other means.

OBSERVATIONS

As AHRQ has implemented the Effective Health Care program, we have some significant observations:

Priority setting: It is important to set clear priorities that meet the most pressing needs of health care decision makers. Therefore, end users and stakeholders must continuously provide input through an open and transparent process.

Framing the research questions: Research must track closely with how clinicians and patients make health care decisions every day. There was much discussion within HHS about how to approach these priorities. During our discussion of research on diseases or conditions, for example, we debated specific questions about treating diabetes and heart disease, and whether our research should center on particular medications and interventions, such as stents or proton pump inhibitors.

We decided to take a disease- and condition-based approach because, at the end of the day, that is how health care decisions are made. A patient comes to the health care system with a condition or disease, and all decisions, including how best to treat it, follow.

This decision to use a disease- and condition-based approach to priorities embodies this perspective. It is also very important to recognize the importance of revising findings frequently to incorporate new evidence that may change the conclusions of what works best and for whom.

Balancing benefits and harms: Comparative effectiveness research must provide information on benefits and harms of a particular medication or intervention. Evaluating the balance of harms and benefits is a critical

component of informed decision making. Few interventions are risk free, and for many chronic conditions the therapeutic goal is management of symptoms and disease state rather than cure. Often times, the decision comes with some assumption of harm—by both patient and clinician—but with the understanding that the benefits are worth that risk.

Research is a means, not an end: The ultimate goal of our research efforts is the development timely, relevant information for decision making. This requires us to go beyond the products of traditional research, namely scholarly articles, and translate findings into language and formats that are appropriate for different audiences. Creating evidence and information that is not useful and accessible, or that does not take advantage of the latest communication technologies and vehicles, is a missed opportunity.

Trust as a process, not a structure: As has been stated, comparative effectiveness research can be a risky business. Therefore, it is important that there be a level playing field among stakeholders. AHRQ's Effective Health Care program has adopted a policy of transparency and inclusion. Manufacturers are notified when a study is begun, are invited to submit relevant studies and data, and have the opportunity—along with any other interested party—to comment on the framing of the specific research questions as well as draft reports. In addition, it is clear that the program's success is dependent on effective collaboration with scientists from industry as well as academia.

The question of trust also extends to the integral role that patients play in research. Although government and the private sector pay for research, patients assume the risks and benefits of enrolling in clinical trials and other studies. A question that is the subject of debate is whether study findings can ethically be kept secret from other researchers and patients themselves (Slutsky 2007). We all need to learn from the knowledge gained in research, but it can be a matter of life and death for patients. We must move to an atmosphere where it is unacceptable to hold back research findings that may have an impact on the care that patients receive.

In conclusion, the U.S. health care system is poised to take advantage of advances in science and health information and communications technology in ways that have previously only seemed like something out of science fiction.

The need for valid, reliable, and accessible information on the comparative benefits and potential harms of treatment options has gained an urgency due to recent policies to promote the adoption of interoperable health IT, continued expansion of diagnostic and treatment options, increased consumer interest in health and health care decisions, and broad interest in improving value.

AHRQ's Effective Health Care program is a model for this vision: A transparent, participatory approach that is driven by the needs of users and encourages broad engagement of stakeholders to mitigate any expected controversies and to expand opportunities for diffusion of findings of comparative effectiveness research.

The Effective Health Care Program represents a foundation in which a larger investment in comparative effectiveness can be built.

REFERENCES

- AHRQ's Research Efforts in Comparative Effectiveness. Statement before the U.S. House of Representatives Subcommittee on Health Committee on Ways and Means by Carolyn M. Clancy, June 12, 2007. Agency for Healthcare Research and Quality, Rockville, MD. Available at <http://www.ahrq.gov/news/sp061207.htm>
- Budget Estimates for Appropriations Committees, Fiscal Year 2008: Performance Budget Submission for Congressional Justification. February 2007. Agency for Healthcare Research and Quality, Rockville, MD. Available at <http://www.ahrq.gov/about/cj2008/cjweb08.htm>
- Clancy, C. M., J. R. Slutsky, and L. T. Patton. 2004. "Evidence-Based Health Care 2004: AHRQ Moves Research to Translation and Implementation." *Health Services Research* 39 (5): xv–xxiv. doi:10.1111/j.1475-6773.2004.00286.x.
- H.R. 2148: The Enhanced Health Care Value for All Act of 2007, Introduced May 7, 2007.
- Public Law 108–173: Medicare Prescription Drug, Improvement, and Modernization Act. 2003: Sec. 1013.
- Serota, S. P. Statement on Ways and Means Hearing to Examine Comparative Effectiveness Research. Blue Cross and Blue Shield Association, June 12, 2007. Available at <http://www.bcbs.com/news/bcbsa/statement-on-ways-and-means-hearing-to-examine-comparative-effectiveness-research.html>
- Slutsky, J. R. "Moving Closer to a Rapid-Learning Health Care System." *Health Affairs* 2007; 26 (2): w122–4.
- Wilensky, G. R. 2006. "Developing a Center for Comparative Effectiveness Information." *Health Affairs* 25 (6): w572–w585. doi: 10.1377/hlthaff.25.w572.