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## Government-Funded Comparative Effectiveness Research

### Background

Patients, physicians, payers, policy-makers, and manufacturers share an interest in taking steps to ensure that there is adequate and accurate information to inform health decision-making concerning the effectiveness of medical interventions. This information can focus on evaluation of the effectiveness of particular drugs, medical procedures or technologies in detecting, treating, or managing diseases. Increasingly, studies in this area try to assess the relative effectiveness of alternative therapies. Significant research has already been performed in this area, sponsored by a variety of agencies and institutions in both the public and the private sectors. A number of policy-makers, however, have recently expressed interest in establishing a national entity or initiative focused on comparative effectiveness research as a solution for reducing the growth in health care costs.

Many factors contribute to rising health care costs including medical errors, outdated and inefficient health care delivery systems, paperwork burdens, and waste, fraud, and abuse within the system. Poor quality of care is estimated to account for one-third of all health care costs<sup>1</sup> and medical errors alone are estimated to add \$38 billion dollars annually to health care spending.<sup>2</sup> Improvements in administrative or health care delivery processes, such as adoption of electronic medical records, greater use of information technology tools to reduce medical errors, alternative options to reduce unnecessary emergency room visits and improved discharge planning would reduce the growth in health care costs. Efforts to reduce health care fraud, abuse, waste, and errors would further reduce the growth.<sup>3</sup>

By contrast, the appropriate use of medical devices and other advanced medical technologies often reduce health care costs by improving outcomes and minimizing complications. Examples include technology to control blood glucose levels that reduces diabetic complications such as retinopathy and peripheral neuropathy; knee replacement surgery resulting in improved quality of life and reductions in nursing home and custodial care costs; imaging technology leading to earlier detection of lung cancer; and minimally invasive breast biopsies that significantly reduce costs and complications enabling women to resume normal activities in half the time.

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1 The Midwest Business Group on Health, "Reducing the Cost of Poor-Quality Care Through Responsible Purchasing," 2002.

2 The Institute of Medicine, *To Err Is Human*, 2000.

3 CMS reported that in 2006, 4.4% of Medicare claims payments (\$10.8 billion) were improper and the HHS Office of Inspector General reported that for FY 2006, it had obtained \$38.2 billion in savings and expected recoveries.

While a number of organizational models are being discussed, many envision an entity that would perform comparative effectiveness studies of alternative interventions. Such studies could range from reviewing existing medical literature to sponsorship of various types of clinical trials or studies. The conduct of such trials or studies would likely be lengthy and costly; with clinical trials requiring the involvement of large numbers of patients.

## **Key Principles**

AdvaMed is strongly committed to the principles of evidence-based medicine and we support comparative effectiveness research as a means to improve clinical outcomes and promote access to quality of care. Sound comparative effectiveness research can be used to assist patients and physicians in medical decision-making by identifying the relative advantages and disadvantages of alternative means to prevent, diagnose and treat disease, including non-treatment as a potential option.

For any entity or initiative involving government-funded comparative effectiveness research, AdvaMed believes that the following principles should be applied to ensure that comparative effectiveness research is carried out appropriately.

- *Patient-centered care and independent professional medical judgment.* Comparative effectiveness research, like all evidence of clinical effectiveness, should inform medical decisions, not replace medical judgment with national treatment formulas. The objective of comparative effectiveness research should be to provide better evidence for physicians and patients to use in making individual clinical decisions. It should enhance, not hinder, the physician's ability to exercise independent professional medical judgment in providing care to their patients.
- *Protecting patient access.* Comparative effectiveness research should not be used by Medicare, insurance companies, or other public or private payers to deny coverage. Comparative effectiveness research typically analyzes which medical intervention, on average, is usually more effective across a population. The intervention that is "generally best," however, may not be best for an individual patient. We believe that protecting patient access to optimal individual patient interventions is paramount. As a result, the entity should inform patients and physicians, but neither make recommendations about coverage or benefits, nor make coverage or benefit decisions.
- *Supporting advances in health care service delivery.* Comparative effectiveness research should include research involving health system changes that affect the management and delivery of health care items, services, and procedures. These include innovations in insurance benefit designs, adoption of electronic medical records, greater use of information technology tools to reduce medical errors, improved discharge planning to allow patients to return to their home or a less intensive setting, when medically appropriate.



- *Setting priorities.* The comparative effectiveness research agenda must be prioritized and designed with pre-stated objectives, research questions, and stakeholder input. The focus of the agenda and resources should be on areas that have major clinical significance and will have the greatest return on investment for the health care system. The agenda development process must be open with priorities developed in consultation with practicing physicians, patients, manufacturers, and other stakeholders to ensure relevance to real world clinical decision-making.
- *Robust databases and analysis.* It is critical that effectiveness be evaluated over a period of time that is appropriate for the specific intervention being evaluated. The length of the episode of care should be determined on a case-by-case basis using clinical expertise to determine the time period over which all relevant benefits and other factors accrue. The length of the episode should not be set arbitrarily – at 30 or 60 days, or 1 year. In addition, it is essential that any database that is used to assess the effectiveness of an intervention include robust data on that particular intervention. This approach will result in a more informed and robust analysis of a given technology or intervention.
- *Studying clinical effectiveness only.* Patients should have access to the interventions that are best for them. Consequently, any government-funded comparative effectiveness research initiative should study clinical effectiveness only. Such research should be used to inform medical decision-making. By focusing government resources on well-designed clinical effectiveness research, quality of care should improve and ultimately should have a favorable impact on overall efficiency in the health care system.
- *Recognizing the process of medical device innovation.* Medical device technologies (both therapeutic and diagnostic products) pose a difficult challenge for producing timely and accurate comparative effectiveness information. Device innovation is evolutionary, and the effectiveness of a particular product is dependent on physician and other health care professional training, experience, and skill. Therefore, research that is conducted too early may quickly become dated as the technology matures and physicians and other health care professionals gain training, experience, and skill. Consequently, a snapshot of comparative effectiveness that involves a particular device at a specific time may incorrectly state the relative effectiveness of an intervention using the device. Accordingly, studies on the comparative effectiveness of devices should consider the effect of training and experience upon outcomes, should be applicable to the current generation of technology, and should only be conducted when the technology has an experience base and is widely available and mature.
- *Transparency and stakeholder input.* Any new structure or initiative set up to conduct comparative effectiveness research must adhere to a process that is conducted in an open and transparent fashion that incorporates stakeholder input. Openness and transparency in all aspects of research—including the determination of research priorities, establishment of the research methodology, and opportunity to comment on the proposed findings through a formal peer review process—will enhance the credibility and strength of the ultimate conclusions of the research. The stakeholders involved should include patients, physicians, hospitals, and experts from the medical device and diagnostics industry, among others. Governance of any public-private entity should include representation of all stakeholders.

## Additional Considerations

- *Defining quality and benefit appropriately.* Comparative effectiveness research should study interventions in a comprehensive fashion and should be tailored to the specific intervention being evaluated. For example, diagnostic technologies pose particular challenges for these types of studies. Because diagnostics are used to inform clinical decision-making, such technologies should be evaluated based on their impact on patient care management. Moreover, comparative effectiveness research should consider including in its analysis of evidence the influence of the following: health-related quality of life (including disability reduction, functional status, reduction in pain, and overall patient satisfaction); work loss and productivity loss; patient adherence; patient preferences and lifestyle choices; symptom control; reduction in medical/medication errors and enhancement of patient and healthcare worker safety; and estimated long-term outcomes (which may result long after a clinical trial has ended).
- *Supporting personalized medicine.* To better enable personalized medicine, comparative effectiveness research findings should be used as a reference, not a mandate, for individual treatment decisions. As scientific advances in technology foster the ability to deliver personalized medicine, independent professional medical judgment and protection of the doctor-patient relationship will become even more crucial. Genomic and proteomic analysis, health information technology, and other advances in health care have the potential to promote tailored treatment decisions for each individual patient's unique needs, thereby saving patients from unnecessary care and saving the health care system from the expense of trial-and-error approaches to therapy.
- *Communication of findings and conclusions.* Research findings should be communicated in a fashion that clearly acknowledges any limitations of the research and underlying data. Armed with the knowledge of which conclusions can and cannot be drawn from a given study or systematic review, patients and physicians will be able to use the research findings appropriately for individual diagnosis and treatment situations. Details regarding the assumptions and data sources should also be readily available. At the same time, there should be a system for assuring that health professionals making decisions are aware of the findings of comparative effectiveness studies.
- *Congressional oversight.* Any government funded comparative effectiveness research initiative, whether conducted through existing agencies or a newly formed organization, should be subject to Congressional and executive branch oversight.