

LEARNING WHAT WORKS BEST

THE NATION'S NEED FOR EVIDENCE ON COMPARATIVE EFFECTIVENESS IN HEALTH CARE



IOM ROUNDTABLE ON EVIDENCE-BASED MEDICINE

– *An Issue Overview* –

APPENDIX SEVEN

Commissioned analysis: The Business Case for Comparative Effectiveness Research

Michael Chernew

Professor, Department of Health Care Policy
Harvard Medical School

Mark Fendrick

Professor, Department of Internal Medicine
University of Michigan Medical School

The IOM Roundtable on Evidence-Based Medicine Issue Overview on Comparative Effectiveness Health Care can be found at:

[http://www.iom.edu/Object.File/Master/43/390/Comparative%20Effectiveness%20White%20Paper%20\(F\).pdf](http://www.iom.edu/Object.File/Master/43/390/Comparative%20Effectiveness%20White%20Paper%20(F).pdf)

INSTITUTE OF MEDICINE

OF THE NATIONAL ACADEMIES

APPENDIX SEVEN

THE BUSINESS CASE FOR COMPARATIVE EFFECTIVENESS RESEARCH *A COMMISSIONED ANALYSIS*

Michael Chernew

Professor, Department of Health Care Policy
Harvard Medical School

Mark Fendrick

Professor, Department of Internal Medicine
University of Michigan Medical School

March, 2007

Acknowledgement: We thank David Helms, Sean Tunis, Peter Juhn, and reviewers from the Institute of Medicine for their helpful comments.

Although the United States spends a far greater amount on health care services when compared to other countries, it is well documented that Americans underachieve in terms of health measures such as life expectancy and infant mortality, as well as other vital health statistics. For instance, in a 2002 ranking of twenty-three industrialized countries, the United States tied for last with 4 other countries on healthy life expectancy at age sixty. Similarly, the US's infant mortality rate ranked last, with rates more than double the average of the three leading countries (France, Japan, and Spain).[1] While some of these discrepancies may be explained by genetic and social factors, it is well accepted that inefficiencies in clinical care – which medical services are used and to whom they are provided – contribute substantially to the discrepancy, particularly the extra spending. Systemic factors in the organization, financing, and delivery of care are part of the problem. The mere fact that Americans consistently under-use medical interventions rigorously proven to provide important health benefits and, at the same time, use substantially more services of unproven value, warrants a careful reassessment of the evidence base that informs medical decision making as well as a payment system that likely rewards inefficient behavior.

Given the complexity of clinical medicine and the financing system, the demands on the evidence base are large. When assessing the value of a specific clinical intervention, the natural tension between quality improvement and cost containment compels a transparent discussion of not only the clinical advantages a certain service may provide, but also the economic ramifications of its use. Moreover, it must be acknowledged that the value of a specific medical intervention will vary across patient groups (e.g., colonoscopy for a 55-year-old woman with a first-degree relative with colon cancer as compared to a low-risk 35-year-old woman). Thus effectiveness is not an attribute purely of the intervention, but of both the intervention and the patient. While acquiring the scientific evidence to improve medical decision making may seem a daunting task, an investment in a clinical research agenda with the specific aim of determining the right medical intervention for the right person at the right time is critical to ensure a more efficient system.

Comparative effectiveness research (CER) refers to the clinical and/or economic evaluations of specific medical interventions (including pharmaceuticals, medical devices and medical procedures)

relative to other available alternatives for a selected clinical indication. CER is very broad. In some cases, it may compare two similar approaches to a particular clinical issue (drug A to drug B, or procedure A to procedure B). In other cases, it may compare available – and dissimilar – clinical approaches (e.g., watchful waiting, radiotherapy and surgery for prostate cancer) to determine the optimal management of a specific clinical problem. CER typically will focus on realistic decisions confronting patients and their clinicians in actual practice, and thus seldom compare interventions to placebo controls. Because of this focus on effectiveness as opposed to efficacy, these investigations will likely rely on both prospective trials and observational data to determine relative value in real world settings, in contrast to the carefully manipulated environment of a controlled clinical trial.

Many countries other than the United States already support a centrally coordinated CER structure, typically financed with public funds. For example, the United Kingdom's National Institute for Health and Clinical Excellence (NICE) produces national guidance on the appropriate use of health technologies and the treatment and prevention of ill health.[2] In the United States, federally funded agencies such as NIH and AHRQ support CER as a part of their established practices, and several for-profit and not-for-profit entities currently engage in CER to varying degrees. However, these efforts are modest when compared to the \$2 trillion annually spent on health care. Although estimates are difficult to obtain, the Coalition for Health Service Research reports that of the \$31.3 billion spent by the federal government in 2005 for health research, only \$1.5 billion (4.8%) was devoted to health services research, of which only a small fraction is likely directed to CER. AHRQ received only \$15 million for CER as part of the Medicare Modernization Act. Moreover, there is little to no coordination among CER efforts.

A greater investment in CER may yield clinical and/or economic benefits. The clinical rewards stem from the belief that CER will support efforts to direct clinical practice towards interventions that yield superior outcomes and away from services that provide little or no clinical benefit. In some cases, evidence suggests that greater benefit can be achieved at an incremental cost. In these cases, policy makers and private sector managers must explicitly acknowledge the tradeoff between better health and medical spending when debating potential solutions to our system's inefficiency. Determining what level of spending should be established for services that lead to improvements in health is one of the most challenging tasks for US decision makers. Several other countries such as Australia, Great Britain, and Canada use measures of incremental cost per health outcome gained in their decisions regarding the allocation of health care resources. For several political and operational reasons, such an approach is unlikely to be adopted in the US in the near future.

Since the over-utilization of unnecessary or unproven medical services is rampant in the US, economic benefits may accrue if CER is able to reduce utilization of these services. CER may also produce cost savings by increasing the use of those services where the clinical benefits are sufficient to reduce spending on adverse outcomes that may have otherwise occurred. There is also potential to enhance efficiency through the identification of clinical approaches that achieve the same clinical outcomes with the utilization of less costly interventions (e.g., laparoscopic versus open cholecystectomy).

This paper examines the potential for CER to enhance the efficiency of the health care system. Because CER is merely a tool, the impact of CER ultimately depends on how it is used, whether it be used to inform decision makers as part of efforts to improve the way markets function, or implemented in specific clinical areas such as guideline development, or in a broader application such as health benefit design. No matter how CER is utilized, it is imperative that outputs from

CER address both the clinical and financial effects, since in some instances improvements in health will require incremental expenditures.

Since there is little argument that the potential clinical and economic impact of any health care reform proposal will depend on the knowledge base that guides practice, such an effort is worthwhile regardless of the approach taken to reform our inefficient system. For example, consumer-driven health care initiatives require patients to know about the clinical and economic ramifications of their medical care choices. Centralized delivery systems require administrators to understand the effects of different clinical options to optimally allocate resources. Other initiatives, such as payment reform or pay-for-performance programs, also require a better understanding of the merits of alternative clinical strategies. Even investments in infrastructure, such as information technology, cannot achieve their full potential without the detailed clinical and economic knowledge provided by CER.

1. Inefficiency in the System

The potential for CER to enhance efficiency depends on the underlying amount of inefficiency in the current health care system. Inefficiency arises when clinical goals could be more efficiently achieved by use of other services, or no services at all. CER inherently addresses the inappropriate use of medical services. Three types of inappropriate use are typically discussed. First, overuse represents the delivery of services that provide no (or minimal) clinical value. In certain cases of overuse (e.g., the use of antibiotic therapy for a viral infection), delivery of these services may result in adverse clinical events and/or a further increase in spending above the costs of the service itself (e.g., patient-level side effects due to allergic reactions as well as societal costs of future antibiotic resistance). Elimination of this type of inappropriate overuse represents the most direct way for CER to achieve cost savings.

A second form of inefficiency, misuse, refers to the use of certain effective services in situations where they are not clinically indicated. This pertains to the aforementioned point that the value of specific medical services differs substantially depending to whom and when they are delivered, and we will consider this a subset of overuse.

Third, underuse represents the failure to deliver appropriate services that attain clinical benefits. Elimination of underuse may not save money in most instances, but still may be perceived as an efficient use of expenditures. However, if the provision of appropriate services can prevent clinical deterioration that would result in greater spending over an extended period (e.g., use of certain medications after a heart attack), savings may be possible.

a) **Overuse.** Overuse in the American Health Care system takes many forms. In some cases, it is the use of an expensive treatment choice within any given treatment strategy. This may be the use of an expensive medication when a cheaper medication is available, or an expensive imaging procedure when a less expensive alternative could be used. A notable example of this behavior was the widespread use of the more expensive cyclooxygenase-2 selective non steroidal anti-inflammatory drug (COX-2 NSAID) for pain relief in patients without risk for gastrointestinal complications. Despite several studies demonstrating no advantages in terms of pain relief when compared to older, less expensive agents, this novel class of agents became the treatment of choice. Similarly, the use of more expensive angiotensin receptor blockers (ARBs) to treat hypertension might not be indicated in patients for whom angiotensin converting enzyme (ACE) inhibitors are effective.

In other cases, overuse occurs when a more expensive treatment strategy is chosen when a less expensive strategy exists, or when a procedure or test is not necessary. For example, in some cases choice of a surgical procedure is more expensive than an alternative pharmacologic intervention (e.g., fundoplication versus proton pump inhibitor therapy for gastroesophageal reflux disease). In other cases, the reverse may be true. In some cases, the use of a diagnostic test could reduce overall costs (e.g., T-wave alternans testing prior to implantable coronary defibrillator insertion), and in other cases diagnostic tests may be unnecessary (e.g., upper gastrointestinal endoscopy in suspected ulcer disease).

In analyzing overuse, it is important to recognize that in some cases waste occurs because the same outcome can be achieved with less expense. In other cases, the more expensive approach provides marginally better outcomes at a much higher cost, in which case the more expensive choice is deemed not cost effective.

Assessing the appropriate strategy is complex. The effects of a treatment strategy may accrue over a long time horizon, and proper analysis will incorporate the costs of complications at a later date. Strategies that seem less expensive at first assessment may in fact be more expensive if they entail more complications over a lifetime. Moreover, costs beyond those of the medical care system, such as effects on worker productivity, should be included.

A second complexity arises because patients are heterogeneous. The efficient choice for one patient may not be efficient for another. For example, the use of COX-2 selective NSAIDs may be appropriate for those patients in need of pain relief who are also at high risk of gastrointestinal complications of traditional NSAIDs (despite the possibility of increased risk of coronary adverse events), whereas this same class of medication is likely to provide no better (and perhaps worse) health outcomes at a higher cost for patients at low gastrointestinal risk but high risk for cardiovascular complications. Often, procedures appropriate in some patient groups diffuse into others, generating overuse due largely to this heterogeneity of risk and benefit.

One major challenge for CER is to assess how to get the right service to the right patient at the right time. A similar critical challenge for the health services research community is to devise systems to limit use to the set of patients to which they provide value, thereby eliminating overuse. Just as the fundamental tenet of clinical medicine is *primum non nocere* “first do no harm,” in today’s complex environment this principle should extend beyond the clinician-patient relationship to health systems operations.

There are many estimates of waste in the American health care system associated with the excessive use of medical interventions. These are documented by several broad categories of literature.

i. Geographic variation: First, the small-area variations literature documents variation in use across geographic regions, controlling for clinical differences. For example, small area analysis, presented in the Dartmouth Atlas of Health Care, compares the use of resources, practice patterns, and spending levels across 306 hospital referral regions to draw inferences about the quality and cost of care provided.[3] Such studies have revealed discrepancies in care for conditions such as hip fracture, colorectal cancer, and acute myocardial infarction.

Fisher et al looked at end-of-life care spending to examine costs and outcomes. They found that residents of high-spending regions received 60% more care, but did not have better quality or outcomes of care.[4] Each 10% increase in regional end-of-life spending was associated with 0.3% – 0.12% increase in risk for death, depending on the original condition.[5] Similarly, cardiac bypass surgery rates exhibit about a fourfold range of variation. These rates are strongly correlated with the numbers of per capita cardiac catheterization labs in the regions but not with illness rates as measured by the incidence of heart attacks in the region.[6]

In another study, mortality rates and quality of life measures were compared for patients undergoing coronary angiography in Texas, where the utilization of the procedure is high (45%), to mortality rates for similar patients in New York, where utilization is low (30%). After adjusting for case mix differences, the researchers found no health advantages associated with greater utilization, suggesting that savings associated with reduced utilization of the procedure in Texas could be achieved with no deleterious clinical consequences.[7]

One estimate suggests that in aggregate, only 27% of the weighted variation in Medicare spending across regions can be explained by population illness levels.[6] If spending levels in all regions were made to match those in the lowest decile (age, sex, and race adjusted), then Medicare could see savings of up to \$40 billion in 1996 dollars.[8]

ii. Inappropriate use: The second body of research that addresses waste in the system is that which attempts to directly measure how frequently certain medical services are delivered for medically inappropriate indications. Results from this literature often demonstrate high levels of inappropriate use. For example, a 1993 study of members of seven managed care organizations found that about 16% of hysterectomies performed were judged to have been clinically inappropriate, and 25% of the patients underwent hysterectomy for uncertain indications.[9] A more recent study (2000) on hysterectomies found more dramatic results. Among hysterectomies performed in a capitated medical group in Southern California, 70% of cases were judged to have been inappropriate, according to RAND appropriateness criteria. Of the 497 women studied, 71 had hysterectomies for conditions covered by three recent ACOG criteria sets. The recommendation for hysterectomy was judged inappropriate for 53% of that subset by the RAND criteria and 76% of those by the ACOG criteria.[10]

In other cases, the rates of inappropriate use are relatively low, but there is a wide range of situations in which appropriateness is uncertain, which demonstrates the need for a stronger evidence base. For example, in one study, 4% of coronary angiographies performed at fifteen hospitals in New York State were rated inappropriate; another 20% were rated uncertain. The rate of inappropriate use varied from 0% to 9% among hospitals, but the difference was not significant.[11] In another study, 4% of percutaneous transluminal coronary angioplasty (PTCA) performed at fifteen hospitals in New York State were rated inappropriate; another 38% were rated uncertain. The inappropriate rate varied from 1% to 9% by hospital, the uncertain rate from 26% to 50%.[12]

Trends toward inappropriate and uncertain use appear in other clinical areas as well. Reviewing cases of new-onset chest pain not due to myocardial infarction at one of five LA-area hospital emergency departments revealed that 7% of those who received some form of diagnostic cardiac testing had tests that were judged to be inappropriate. A literature review on cases of metastatic renal cell cancer (MRCC) rated 46.9% of treatments as inappropriate and 25.8% as uncertain.[13] A review of Medicare patients in three geographic areas revealed that 32% of the sample had carotid

endarterectomy for inappropriate reasons, and 32% for uncertain reasons.[14] 17% of diagnostic upper gastrointestinal endoscopy procedures for Medicare patients were performed for inappropriate indications, and 11% were performed for uncertain indications.[15] In cases of hospital use, 23% of admissions were judged to be inappropriate and an additional 17% could have been avoided by the use of ambulatory surgery.[16]

These studies often examine a specific intervention (e.g., upper gastrointestinal endoscopy, or percutaneous coronary angioplasty) and evaluate the usefulness in a number of clinical indications. Most of the appropriateness research focuses on high unit cost services. However, significant expenditures associated with overuse may accrue from inappropriate utilization of low unit cost services if they are used in sufficient volume (e.g., routine blood testing, imaging procedures). Moreover, many of the studies cited above are based on the 1980s. The more recent small area variations literature suggests that substantial inappropriateness likely still exists, but much more work is needed in the area if we are to better understand, and address, the inefficiencies in the system.

These findings of substantial variation in practice patterns and often large rates of inappropriate use highlight the challenge that the merit of a specific medical intervention depends on the precise reason for use. Thus, in most situations, detailed patient-specific information is required before reporting whether the use of a drug, test, or device is worthwhile.

It is important to recognize that medical services are not always appropriate or always inappropriate. Consider an example in the area of diagnostic imaging, radionuclide cardiovascular imaging (RNI). This is but one type of diagnostic imaging, but understanding the appropriateness of imaging as a whole is crucially important. Diagnostic imaging services reimbursed under Medicare's physician fee schedule grew more rapidly than any other type of physician service. Between 2000 and 2005, spending doubled from \$6.6 billion to \$13.7 billion.[17] In 2005, the American College of Cardiology Foundation convened a technical panel to assess the appropriateness of RNI for 52 indications.[18] Of the 52 indications, 13 were deemed inappropriate, 27 appropriate, and for 12 the appropriateness was uncertain. Moreover, there was not even consensus on all of the indications for which RNI was deemed appropriate. For example, for 6 of the 27 indications deemed appropriate, there was strong disagreement among the panelists about that designation. Much more research is needed to reduce the level of clinical uncertainty and move the system towards efficient practice patterns.

However, CER will not be sufficient to eliminate overuse. Even when identified, system factors and the complexities of care limit the ability of the system to eliminate the waste. Research on these system factors, including patient- and system-oriented interventions such as benefit design and clinician/hospital reimbursement, will be needed to complement CER and allow development of the systems needed to realize the potential offered by CER.

b) Underuse. Paradoxically, while overuse in the health care system is common, underuse of medical services rigorously determined to provide substantial clinical benefit is also widespread. While the small area variation discussion commonly focuses on overuse, similar aggregate level outcomes in high expenditure areas and low expenditure areas imply that some of the small area variation may be due to underuse. For example, among patients with heart attacks who were considered "ideal candidates" for beta-blockers, those who actually received the needed drug ranged from 5% to 92% of patients among the 306 Dartmouth Atlas Hospital Referral Regions (HRRs).[6]

A substantial portion of underuse reflects the failure of individuals or their physicians to use preventive services, or to manage their chronic illnesses as the scientific evidence would recommend. CER is needed to improve our ability to identify when variation represents underuse and when it represents overuse so the system can respond appropriately. However, as with overuse, CER will not be sufficient to eliminate underuse. While the clinician-patient relationship plays a critical role to this shortcoming, systemic effects such as access to care, benefit design, and ability to pay are also likely contributors, and more research examining these factors will be needed to improve the ability of the system to integrate CER findings into practice.

2. Effects of inefficiency on key stakeholders

Inefficiency in the health care system, particularly that which leads to unnecessary expenditures, affects all stakeholders. Both overuse and underuse reduce the value of the resources devoted to the health care system. The enormous incremental costs associated with this inefficiency are borne throughout the system.

a) **Individuals.** Individuals, whether or not they use the system, pay for these inefficiencies in several ways and are unmistakably worse off. First, in some cases, individuals pay out of pocket for services (e.g., total body imaging scans) that provide little value in terms of clinical outcomes. Second, the financial costs associated with waste are reflected in higher health care premiums. These are paid by workers directly, or indirectly because higher health care costs lead employers to pay lower wages.[19] Third, higher costs for public programs are financed by taxpayers. The costs of the largest public program, Medicare, rose 8.9% to \$342.0 billion between 2004 and 2005.[20] Furthermore, projections suggest Medicare will grow at an annual rate of over 9% between 2005 and 2015.[21] The growth of Medicare spending will represent a serious burden for taxpayers and a significant challenge for policymakers. It is well established that the tradeoff between access to medical care and how to pay for it is a complex and extremely political issue.

Fourth, high health care costs are also associated with declining rates of health insurance coverage.[22] To the extent that greater waste leads to fewer covered individuals, those that are un- and underinsured must bear greater financial risk and suffer the consequences of diminished access to valuable care in the event such care is needed.

Finally, inefficiency generates additional adverse consequences for patients already engaged in the system. Specifically, the overconsumption of care often entails clinical risk as well as financial costs. Over the past decade, the “patient safety” movement has brought to light the extent of the clinical and economic ramifications of avoidable medical errors. For example, hospital-acquired infections are estimated to be responsible for \$3.5 billion to \$5.7 billion in excess health care costs each year.[23, 24] Under-utilization also generates suboptimal clinical outcomes as patients forego utilization of important services.

b) **Employers.** The clinical and financial effects of inefficient care delivery on other stakeholders are more complex. To the extent that employers bear a large fraction of the costs associated with inefficiency, they are adversely affected. As mentioned above, standard economic models supported by empirical evidence suggest that over time, employers shift the costs of higher health care spending to workers in the form of lower wages. However, in the short run, employers (or the shareholders) may bear some of the costs of inefficiency. Moreover, the ability to shift cost to workers is limited for retiree expenses, suggesting that shareholders will bear the costs of inefficiency

for this population of workers. Employers may also bear some of the administrative costs associated with managing health care benefits in an environment of rising costs and considerable inefficiency.

c) Health Insurers

The fiscal implications of inefficiency on insurers are also complicated. To the extent that cost increases can be anticipated, they may be included in premiums. However, as premiums escalate, the demand for coverage may be dampened, suggesting that on balance insurers will find it challenging to remain profitable in a rising cost environment over the long run. Yet with the challenge comes opportunity. If insurers can develop ways to address the problems of inefficiency in the health care system, substantial profit opportunities may arise.

d) Providers of Health Care Services

Providers of health care services – especially those whose income is related to productivity, not quality of care – may be one stakeholder group that benefits from inefficiency. Since one group's expense is another's revenue, the payments for unnecessary interventions are income for the providers of those services. Thus, while no physician or hospital may intentionally, or even knowingly, provide unnecessary services, they likely reap some financial gain from the services delivered, necessary or not. The magnitude of this effect for any one provider depends on the extent to which they deliver unnecessary care. Providers of necessary care would not be adversely affected by reductions in the use of unnecessary services.

Reductions in the use of unnecessary care may offer indirect benefit to providers in the long term. Specifically, higher costs lead to fewer people with coverage. This may place a burden on providers who are increasingly called on to provide uncompensated care. Providers may also benefit from any reductions in inefficient care because they may find this type of cost containment preferable than other approaches (such as fee reductions).

e) Manufacturers

The impact of inefficiency (and efforts to reduce inefficiency) on manufacturers is much the same as that on the providers of those services. Any reduction in utilization may be a reduction in revenue, but the effects will target low-value or unnecessary services. Manufacturers that have the potential to make important clinical advances can thrive in a low waste environment. Moreover, relative to other cost containment efforts that may impact manufacturers, efforts to reduce unnecessary use of certain medical products may be preferable.

3. Uses of CER

Discussions of CER frequently focus on the use of these evaluations to assist in development of practice guidelines or in coverage/payment decisions. While CER could be used in these specific endeavors, CER is needed for more far-reaching efforts to improve the efficiency of the health care system. The critical nature of a comprehensive CER agenda arises because of the lack of controlled assessments of available therapeutic options and the substantial amount of patient heterogeneity that exists. Waste generally arises when services that are valuable in some clinical situations are applied to other indications. CER is an essential tool to determine which intervention should be delivered to which person and in what clinical circumstance.

By facilitating improved targeting of both the clinical intervention and the specific patient population, the information provided by CER can benefit key stakeholders, particularly patients and payers. Specifically, by reducing uncertainty associated with which treatment course is most appropriate, CER can decrease the frequency that patients receive inappropriate care, reducing costs and potential for harmful medical errors. Similarly, CER can facilitate efforts to develop coverage policy and design value based insurance packages, which should enhance the return on health care expenditures made by payers – private or public.²⁵ Taking the perspective of the provider, the effects of CER on utilization will depend on both the nature of their product and the incentives in place to use the service. If coverage and reimbursement levels reflect the findings of CER [i.e. payment based on clinical effects not exclusively production cost], providers and manufactures of high value services should find the CER increases their market share. However, the demand for low value services will likely [appropriately] decline. Given that the burden of proof necessary to demonstrate value in the marketplace may intensify, so might the costs to perform the requisite CER studies.

A particular concern for providers is that cost containment efforts designed to eliminate use of unnecessary services often inadvertently lead to restrictions on the provision of needed care. In almost all of the studies that report the appropriate indications for the use of a specified intervention, the appropriateness is ‘uncertain’ in a significant portion of situations. Recall that there are few instances where the use of a specific drug, diagnostic test or procedure is always appropriate or inappropriate. This underscores the need for a CER agenda that is able to measure health and economic impact on a granular level that will ultimately target those specific circumstances when certain interventions should and should not be used.

While the evidence examines both under- and overuse of selected medical services, one cannot accurately predict the net effect of a more efficient system on expenditures. This is related to the tradeoffs of how a better evidence base drives the increased use of more valuable services (and likely increases expenditures) and slows the utilization of low-value interventions (and decreases spending).

Individual CER studies may not always suggest that the least expensive course of action is the appropriate course of action – recall that “the most expensive therapy is the one that doesn’t work.” However, medical culture tends to be driven towards the adoption of new, expensive services, and cost growth has widely been attributed to the development and diffusion of new medical services.[25-27] Therefore, on balance, we would expect that CER would tend to dampen spending to a level below that which would otherwise occur, because the ‘adopt everything for everyone’ mentality would be replaced with an ‘adopt when appropriate’ paradigm. For example, a 2006 study examined whether some stable, high-risk patients with persistent total occlusion of the infarct-related coronary artery should undergo percutaneous coronary intervention (PCI) in addition to receiving optimal medical therapy.[28] Although use of this procedure in such cases was not universal, the authors report an inclination among physicians towards its use. In this case, a randomized trial demonstrated that PCI did not improve clinical outcomes, suggesting resources could be saved by foregoing the procedure. The trend would likely have been towards greater use, and the CER-suggested lower use was medically appropriate.

Since the literature on diffusion of medical technology clearly shows a preference for US clinicians to use new interventions before definitive clinical data are available, one can safely assume that the clinical data provided by a CER agenda will improve the quality of care. However, it should not be

assumed that the completion and implementation of a CER agenda will save money in the short term. The short-term financial consequences will depend on how CER is used and on whether the savings incurred to lower rates of use of low valued interventions will offset the added expenses of the increased use of higher valued services.

While enhancing the health of Americans is a noble goal, we acknowledge that cost containment is an integral and inevitable part of the future health care policy. Without a strong investment in CER, patients and providers are more likely to face unintended “across the board” restrictions on the provision of valuable care because of the fiscal pressures that are being imposed on public and private health care payers. Whether these are manifested by fewer insured individuals or the underinsurance of those with some type of benefits, CER provides the knowledge base by which providers of high-value services can advocate their continued use using accepted scientific approaches to make their case. The findings of research that directly compares the pros and cons of available treatment options from numerous perspectives will be important for clinical practice, regardless of the cost containment/benefit reform approaches being considered. Cost containment efforts that rely on an improved evidence base are likely preferable to current efforts to drive all practice towards those of the lowest cost. Findings from CER should be used to better target, not limit, care.

The exact mechanisms by which CER will lead to enhanced efficiency will vary based on the level of detail of the data generated by the studies and the ability of the system to implement the findings in everyday practice. On the quality improvement side, similar challenges have been identified in studies examining the suboptimal uptake of evidence-based practice guidelines. From the financial perspective, cost sharing approaches aim to control spending by making patients pay more at the point of service. Most efforts to raise patient out-of-pocket costs have resulted in higher costs across all services (with the possible exception of some preventive health services). It has been demonstrated that financial disincentives are often placed at the patient level, making adherence with evidence-based care difficult. Yet when faced with higher costs, patients often make poor clinical decisions, which in fact could, in some cases, lead to greater overall costs. Thus, the alignment of clinical and financial incentives is a necessary component to insure the attainment of an efficient delivery system. The status quo has been unable to align quality improvement and cost containment initiatives. In fact, in some instances they actually compete with one another, contributing directly to inefficiency.[29]

Such an alignment of incentives is possible in the setting of improved clinical evidence – driven by CER - and health benefit reform. Value Based Insurance Design (VBID) represents a “clinically sensitive, fiscally responsible” approach that advocates keeping patient out-of-pocket payments low on high-value services and raising them on services of no or marginal clinical value. Similar processes can be developed for clinician payment (e.g., payment based on quality of care delivered, not productivity). Implementation of such a scheme, in any form, would require greater CER since the relative value of services would be directly based on the findings. The advantages of such an alignment of clinical and economic incentives are obvious when compared to the current approach of untargeted “across the board” cost sharing schemes, where the rates of both non-essential and essential services are negatively affected by higher out-of-pocket rates. By using incentives to encourage the use of high valued services and discouraging low valued ones, VBID has the potential to achieve marked increases in the efficiency of the health care system.

Supply side oriented health care reform approaches could also benefit from added investment in and coordination of CER. Certainly, coverage policy and clinical guidelines require such knowledge. But other initiatives such as provider education, disease management, or pay-for-performance programs all require an understanding of which services provide value in which settings and how quality and cost metrics can be designed in a clinically meaningful way.

4. *Conclusions:*

Health care cost growth has placed a growing strain on our health care financing system. Although there is no consensus about how we can address the health care cost issue, most stakeholders would probably agree that the resources devoted to health care must be allocated more efficiently. This will entail being able to identify situations when more resources are necessary to overcome the problem of underuse of highly valued services that improve health, as well as when money is being wasted on interventions that do not improve health, or worse, actually produce adverse consequences.

Regardless of the reform approach considered – market-based health savings accounts or a system administered through a single source – enhanced efficiency will require more detailed knowledge about the relative effectiveness of different interventions in specific clinical indications. All vested stakeholders should encourage investment in an infrastructure that prioritizes and undertakes investigations that yield practical information on which services to provide to which patients and when. Our health care system is too complex and too large to be guided without an appropriate knowledge base. Moreover, because innovation in the health care sector is substantial, investment in an infrastructure that would allow the assessment of the clinical and economic impact of new and existing diagnostic and treatment modalities is essential.

Creating this infrastructure will require a substantial investment. For those who consider the upfront investment necessary to create such an infrastructure to be unaffordable, it is imperative to contemplate the costs of the status quo which propagate tremendous inefficiency.

REFERENCE

1. Schoen, C, et al., *US Health System Performance: A National Scorecard*. Health Affairs Web Exclusive, 2006: p. W457-75.
2. Kaufman, JS, et al., *Subcutaneous compared with intravenous epoetin in patients receiving hemodialysis. Department of Veterans Affairs Cooperative Study Group on Erythropoietin in Hemodialysis Patients*. New England Journal of Medicine, 1998. **339**(9): p. 578-83.
3. Fisher, ES and Wennberg, JE, *Health care quality, geographic variations, and the challenge of supply-sensitive care*. *Perspect Biol Med*, 2003. **46**(1): p. 69-79.
4. Fisher, ES, et al., *The implications of regional variations in Medicare spending Part 1: the content, quality, and accessibility of care*. *Ann Intern Med*, 2003. **138**(4): p. 273-87.
5. Fisher, ES, et al., *The implications of regional variations in Medicare spending Part 2: health outcomes and satisfaction with care*. *Ann Intern Med*, 2003. **138**(4): p. 288-98.
6. Wennberg, JE, Fisher, ES, and Skinner, JS, *Geography and the debate over Medicare reform*. *Health Aff (Millwood)*, 2002. **Suppl Web Exclusives**: p. W96-114.
7. Guadagnoli, E, et al., *Variation in the use of cardiac procedures after acute myocardial infarction*. *N Engl J Med*, 1995. **333**(9): p. 573-8.

8. Wennberg, JE and Cooper, MM, eds. *The Dartmouth Atlas of Health Care* 1999, American Hospital Assoc: Chicago.
9. Bernstein, SJ, et al., *The appropriateness of hysterectomy. A comparison of care in seven health plans. Health Maintenance Organization Quality of Care Consortium.* *Jama*, 1993. **269**(18): p. 2398-402.
10. Broder, MS, et al., *The appropriateness of recommendations for hysterectomy.* *Obstet Gynecol*, 2000. **95**(2): p. 199-205.
11. Bernstein, SJ, et al., *The appropriateness of use of coronary angiography in New York State.* *Jama*, 1993. **269**(6): p. 766-9.
12. Hilborne, LH, et al., *The appropriateness of use of percutaneous transluminal coronary angioplasty in New York State.* *Jama*, 1993. **269**(6): p. 761-5.
13. Halbert, RJ, et al., *Treatment of patients with metastatic renal cell cancer: a RAND Appropriateness Panel.* *Cancer*, 2006. **107**(10): p. 2375-83.
14. Winslow, CM, et al., *The appropriateness of carotid endarterectomy.* *N Engl J Med*, 1988. **318**(12): p. 721-7.
15. Kahn, KL, et al., *The use and misuse of upper gastrointestinal endoscopy.* *Ann Intern Med*, 1988. **109**(8): p. 664-70.
16. Siu, AL, et al., *Inappropriate use of hospitals in a randomized trial of health insurance plans.* *N Engl J Med*, 1986. **315**(20): p. 1259-66.
17. *Kuhn, H. Testimony on Payment for Imaging Services under the Medicare Physician Fee Schedule.* July 18, 2006 [cited 2007 January 22]; Available from: <http://www.hhs.gov/asl/testify/t060718.html>.
18. Brindis, RG, et al., *ACCF/ASNC appropriateness criteria for single-photon emission computed tomography myocardial perfusion imaging (SPECT MPI): a report of the American College of Cardiology Foundation Quality Strategic Directions Committee Appropriateness Criteria Working Group and the American Society of Nuclear Cardiology endorsed by the American Heart Association.* *J Am Coll Cardiol*, 2005. **46**(8): p. 1587-605.
19. Gruber, J, *The incidence of mandated maternity benefits.* *Am Econ Rev*, 1994. **84**(3): p. 622-41.
20. CMS Office of Public Affairs. *Press Release: CMS Releases U.S. Health Spending Estimates Through 2005.* January 9, 2007 [cited 2007 January 22]; Available from: <http://www.cms.hhs.gov/apps/media/press/release.asp?Counter=2069&intNumPerPage=10&checkDate=&checkKey=&srchType=&numDays=3500&srchOpt=0&srchData=&keywordType=All&chkNewsType=1%2C+2%2C+3%2C+4%2C+5&intPage=&showAll=&Year=&year=&desc=&cboOrder=date>.
21. Borger, C, et al., *Health spending projections through 2015: changes on the horizon.* *Health Aff (Millwood)*, 2006. **25**(2): p. w61-73.
22. Chernew, M, Cutler, DM, and Keenan, PS, *Increasing health insurance costs and the decline in insurance coverage.* *Health Serv Res*, 2005. **40**(4): p. 1021-39.
23. Burke, JP, *Infection control - a problem for patient safety.* *N Engl J Med*, 2003. **348**(7): p. 651-6.
24. Hollenbeak, CS, et al., *Factors associated with risk of surgical wound infections.* *Am J Med Qual*, 2006. **21**(6 Suppl): p. 29S-34S.
25. Chernew, ME, et al., *Managed care, medical technology, and health care cost growth: a review of the evidence.* *Med Care Res Rev*, 1998. **55**(3): p. 259-88; discussion 89-97.
26. Cutler, DM, *Technology, Health Costs, and the NIH.* 1995, Harvard University and the National Bureau of Economic Research: Cambridge, MA.
27. Newhouse, JP, *Medical care costs: how much welfare loss?* *J Econ Perspect*, 1992. **6**(3): p. 3-21.
28. Hochman, JS, et al., *Coronary intervention for persistent occlusion after myocardial infarction.* *N Engl J Med*, 2006. **355**(23): p. 2395-407.

29. Chernew, ME, Rosen, AB, and Fendrick, AM, *Rising out-of-pocket costs in disease management programs*. Am J Manag Care, 2006. **12**(3): p. 150-4.

The IOM Roundtable on Evidence-Based Medicine Issue Overview on Comparative Effectiveness Health Care can be found at:

[http://www.iom.edu/Object.File/Master/43/390/Comparative%20Effectiveness%20White%20Paper%20\(F\).pdf](http://www.iom.edu/Object.File/Master/43/390/Comparative%20Effectiveness%20White%20Paper%20(F).pdf)