Producing Comparative-Effectiveness Information

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Statement of
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Chairman Stark, Ranking Member Camp, distinguished subcommittee members, I am Mark Miller, Executive Director of the Medicare Payment Advisory Commission (MedPAC). I appreciate the opportunity to be here with you this morning to discuss comparative-effectiveness research. MedPAC constantly seeks improvements in Medicare efficiency, as evidenced in our work improving the accuracy and equity in Medicare’s payment policies, pay-for-performance, coordination of care, and the subject for today—comparative effectiveness. Improving efficiency involves getting better quality with fewer resources and getting more of the right care. One way we recommend to do so is to develop more comprehensive information on the comparative effectiveness of health care services.

The current trends in public and private health care spending are unsustainable. Even though substantial resources are devoted to health care in the U.S., the value of services furnished to patients is often unknown. Many new services disseminate quickly into routine medical care with little or no basis for knowing whether they outperform existing treatments, and to what extent. Increasing the value of health care spending requires knowledge about the outcomes of services. Comparative effectiveness—a comparison of the outcomes of different treatments for the same condition—could help all public and private payers to get greater value from their resources.

There is not enough credible, empirically based comparative-effectiveness information available to patients, providers, and payers to make informed treatment decisions. Comparative effectiveness is a public good because the benefits of the information accrue to all users, not just to those who pay for it. Because it is a public good, a federal role is necessary to produce the information and make it publicly available. Consequently, the Commission recommends that the Congress should charge an independent entity to sponsor credible research on the comparative effectiveness of health care services and disseminate this information to patients, providers, and public and private payers.

Such an entity would:

- Be independent and have a secure and sufficient source of funding;
• Produce objective information and operate under a transparent process;
• Seek input on agenda items from patients, providers, and payers;
• Reexamine comparative effectiveness of interventions over time;
• Disseminate information to providers, patients, and federal and private health plans; and
• Have no role in making or recommending coverage or payment decisions for payers.

There are different ways to carry out a federal role. The Commission prefers a public–private option, reflecting the benefit of comparative-effectiveness information to the government, private payers, and their patients. Funding could come from some public and some private sources or alternatively from public sources only. An independent board of experts should help develop the research agenda and ensure that the research is objective and methodologically rigorous. The entity’s primary mission would be to sponsor studies that compare the clinical effectiveness of a service with its alternatives. This research may involve synthesizing existing analyses or sponsoring new analyses. We emphasize that the entity would not have a role in how payers apply this information—that is, coverage or payment decisions. Instead, it would produce and disseminate comparative-effectiveness information to payers, providers, and patients who would then decide how to use it.

**Context for Medicare payment policy**

Medicare was designed to help ensure access to medically necessary care for the aged and disabled. Many analysts give Medicare credit for improving the economic position of its beneficiaries. Today, however, Medicare and other purchasers of health care in our nation face enormous challenges for the future. One challenge relates to the wide variation in the quality and use of services within our health care system, with quality often bearing no relationship or even a negative relationship to spending. Analysts point to geographic variation in spending as evidence of inefficiency and waste. Although spending is rising, it is not clear that beneficiaries are seeing commensurate increases in the quality of their care or their health. A second challenge is that health care spending in general and Medicare’s spending in particular has been growing much faster than the economy. Forces such as the broad dissemination and use of newer medical
technologies can be beneficial but it can also unnecessarily contribute to higher costs if the innovation is adopted without evidence that it is superior to existing treatments and is used in populations where it is not effective. The forces that are driving these spending trends are common to both public and private payers. Because of these forces, the Commission and others have continually warned of a serious mismatch between the benefits and payments the program currently provides and the financial resources available for the future.

Figure 1 shows the Medicare trustees’ view of the future of Medicare financing. Total expenditures for Medicare will take up an increasing share of the nation’s gross domestic product (GDP) and quickly exceed dedicated financing. In their most recent report, the Medicare trustees project that, under intermediate assumptions, the hospital insurance (HI) trust fund (which finances Part A of Medicare) will be exhausted in 2019. There is no provision to use general revenues to cover Part A services once the HI trust fund is exhausted. Consequently, either those expenditures will have to cease or some new source of financing will have to be found. For other parts of Medicare (Part B and Part D), general tax revenues and premiums automatically increase with expenditures. Those automatic increases will impose a significant financial liability on Medicare beneficiaries, who must pay premiums and cost sharing, and on taxpayers in general. For example, if income taxes remain at their historical average share of the economy, the Medicare trustees estimate that the program’s share of personal and corporate income tax revenue would rise from 10 percent today to 24 percent by 2030.
Figure 1. Medicare faces serious challenges with long-term financing

Note: GDP (gross domestic product), HI (Hospital Insurance). Tax on benefits refers to income taxes that higher income individuals pay on Social Security benefits that are designated for Medicare. State transfers (often called the Part D "clawback") refer to payments from the states to Medicare for assuming primary responsibility for prescription drug spending.

Source: 2007 annual report of the Boards of Trustees of the Medicare trust funds.

Figure 2 shows that between 1970 and 2005, the average monthly Social Security benefit (adjusted for inflation) increased by an annual average rate of 1.6 percent. Over the same period, average supplementary medical insurance (SMI) premiums plus cost sharing and average SMI benefits grew by annual averages of 4.5 percent and 5.9 percent, respectively. In the 2003–2006 period, Part B premium increases offset 20 percent to 40 percent of the dollar increase in the average Social Security benefit. For 2007, the increase in the Part B premium offsets 13 percent of the Social Security benefit increase. Medicare trustees project that between 2006 and 2036,
the average Social Security benefit will grow by just over 1 percent annually (after adjusting for inflation), compared with 3 percent annual growth in average SMI premiums plus cost sharing.

**Figure 2. Average monthly SMI benefits, premiums, and cost sharing are projected to grow faster than the average monthly Social Security benefit**

Policymakers will need to use a combination of approaches to address Medicare’s long-term sustainability. Use of comparative-effectiveness information is one approach, as it has the potential to increase the value of the health care spending that is going to occur. It is possible that comparative effectiveness could reduce spending if, among clinically comparable services, less costly services replace more costly services. Since Medicare heavily influences many aspects of health care, policymakers should keep in mind that the program could play a leading...
role in initiating change. At the same time, broad trends in the health care system affect the
environment in which it operates, and Medicare needs to collaborate with private payers who
face similar pressures from growth in health spending.

**Defining comparative effectiveness**

Comparative-effectiveness analysis evaluates the relative impact of medical services, drugs,
devices, therapies, and procedures used to treat the same condition. Effectiveness means the
outcomes of clinically relevant alternatives provided to patients with diverse clinical
characteristics in a wide variety of practice settings. The outcomes that researchers assess in
comparative-effectiveness studies may include: clinical outcomes, such as mortality and major
morbidity; functional outcomes, such as quality of life, symptom severity, and patient
satisfaction; and economic outcomes, including cost effectiveness.

**The private sector does not systematically produce and disseminate objective comparative-effectiveness information**

In some instances, manufacturers conduct studies assessing the clinical and cost effectiveness of
their products but some researchers have critiqued these studies and raised concerns that these
efforts may not always be objective and available to the public. Researchers have shown that
bias in drug trials is common and often favors the sponsor’s product. Possible sources of bias in
industry-sponsored trials include: (1) the dose of the drug studied, (2) the exclusion of patients
from the study population, (3) the statistics and methods used, and (4) the reporting and wording
of results. Some are also concerned that not all manufacturers disseminate studies that show
negative results of their services and treatments.

Pharmacy benefit managers, health plans, and other large providers (e.g., hospitals) consider a
service’s clinical effectiveness, cost, and cost effectiveness, particularly for their drug
formularies, but do not necessarily make their evaluations public. These groups often focus on
proprietary internal studies related to their health care practices. Few private sector groups
systematically produce clinical and cost-effectiveness information and make it available to the
public. One exception is the Technology Evaluation Center established by Blue Cross Blue Shield Association, which relies on reviewing the existing literature to compare the clinical effectiveness of alternative services and posts it studies on the internet.

Concerns about liability might affect some private plans’ use of cost-effectiveness information in their decision-making process. For example, some health plans reluctantly agreed to cover high-dose chemotherapy with autologous bone marrow transplant for breast cancer partly in response to the threat of litigation, despite its high cost and the lack of evidence that it was effective.

Furthermore, one could argue that comparative effectiveness is a public good because it demonstrates:

- “Nonexcludability”: Once comparative-effectiveness information is publicly available, it is difficult to stop other groups from using the research free of charge.
- “Nonrivalness”: One group’s use of the information does not detract from its use by other groups.

Conducting this type of research is costly and, when it is publicly available, its benefits accrue to all, not just to those who pay for it. Although private plans have some of the data to conduct this research, they lack incentives to support it at the needed levels. Economic theory argues that the private sector will underproduce services (or in this case information) that meet this definition and that a government role is necessary to ensure that a sufficient supply is available.

**Conducting comparative-effectiveness studies is not the primary focus of any federal agency**

Some federal agencies do conduct comparative-effectiveness research, including CMS, the Agency for Healthcare Research and Quality (AHRQ), the National Institutes of Health (NIH), and the Department of Veterans Affairs (VA). However, their efforts are not substantial enough or coordinated enough to affect needed change.
CMS’s efforts

CMS assesses the clinical effectiveness of services when making national coverage decisions. The agency bases these assessments partly on reviewing available literature about the service. In addition, CMS gathers information about a service’s clinical effectiveness through registries and clinical trials for services the agency might not have covered in the past because of insufficient data about the service’s clinical value. In some cases, CMS supplements its research by sponsoring outside groups, such as NIH, to conduct head-to-head trials and AHRQ and the Medicare Evidence Development & Coverage Advisory Committee to conduct and review assessments of the medical and economic implications of the use of health care services.

AHRQ’S efforts

AHRQ compares the clinical effectiveness of alternative treatments under a provision in the Medicare Prescription Drug, Improvement, and Modernization Act of 2003 (MMA) that mandated the agency to conduct and support research with a focus on outcomes, comparative clinical effectiveness, and appropriateness of pharmaceuticals, devices, and health care services. Beginning in 2005, the Congress appropriated $15 million per year for the agency to fulfill its MMA mandate (the MMA authorized up to $50 million for this research effort). To fulfill the MMA mandate, AHRQ has: (1) put processes in place to select topics for analysis, review, and synthesis of the scientific literature, and to obtain input from the public and private sectors; (2) developed the infrastructure to conduct comparative-effectiveness research and translate the information to providers and patients; (3) completed eight studies, with more than 30 studies in progress; and (4) disseminated the research findings to users.

Outside of the MMA mandate, AHRQ has conducted studies examining both the clinical effectiveness and cost effectiveness of services for CMS and NIH. For example, on behalf of CMS, AHRQ assessed the cost effectiveness of fecal occult blood tests.

Conducting comparative-effectiveness research is not AHRQ’s main mission, although the agency’s efforts in this area are significant. Its primary mission is to conduct and sponsor health services research—the multidisciplinary field of scientific investigation that studies how social
factors, financing systems, organizational structures and processes, health technologies, and personal behaviors affect access to health care, the quality and cost of health care, and the health and well-being of the U.S. population.

**NIH’s efforts**

NIH is the largest federal sponsor of clinical trials that compare a therapy to its alternatives—head-to-head trials. For example, NIH and CMS cosponsored the ongoing head-to-head trial comparing more frequent hemodialysis with thrice weekly (conventional) hemodialysis for patients with end-stage renal disease.

**VA’s efforts**

VA also sponsors head-to-head clinical trials and cost-effectiveness analyses specific to its patient population. Since 1994, the VA has required a formal cost-effectiveness analysis from manufacturers of drugs that have small differences in quality but large differences in cost compared with their alternatives. The VA routinely requests manufacturers to submit clinical and economic data and incorporates this information into the drug reviews used in the formulary decision-making process.

**The United States needs to produce more credible comparative-effectiveness information**

The Commission finds that not enough credible, empirically based comparative-effectiveness information is available for patients, health care providers, and payers to make informed decisions about alternative services for diagnosing and treating most common clinical conditions. For private-sector groups, conducting this type of research is costly and, when it is made publicly available, the benefits accrue to all users, not just to those who pay for it. Although several public agencies conduct comparative-effectiveness research, it is not their main focus and their efforts are not conducted on a large enough scale.

Consequently, the Commission recommends that the Congress should charge an independent entity to sponsor credible research on comparative effectiveness of health care services and
disseminate this information to patients, providers, and public and private payers. Other organizations and policy analysts from disparate points of view have reached a similar conclusion, including Blue Cross/Blue Shield Association, America Health Insurance Plans, Gail Wilensky, Marilyn Moon, and Uwe Reinhardt. (Complete bibliographic citations are available in our forthcoming June 2007 report to the Congress.)

**Comparative information could help payers make better policies**

Several ways for both public and private payers to use comparative-effectiveness information in the payment process include:

- Creating a tiered payment structure that pays providers more for those services that show more value to the program;
- Creating a tiered cost-sharing structure that requires lower cost sharing for those services that show more value to the program;
- Not paying the additional cost of a more expensive service if evidence shows that it is clinically comparable to its alternatives; and
- Requiring manufacturers to enter into a risk-sharing agreement, which links actual beneficiary outcomes to the payment of a service based on its comparative effectiveness. This idea requires that manufacturers rebate the payer for services that do not meet expectations for their effectiveness.

Public and private payers might use comparative-effectiveness information to prioritize pay-for-performance measures, target screening programs, or prioritize disease management initiatives. A pay-for-performance program could link providers’ bonuses to the provision of services that are clinically effective and of high value.
More comparative information could help support better decision making by patients and providers

Many new services disseminate quickly into routine medical care without providers knowing whether they outperform existing treatments, and to what extent. For example, a recent study showed that inexpensive diuretics may control hypertension as effectively as expensive calcium-channel blockers.

The FDA’s regulatory process for approving new technologies does not in general generate evidence that shows a service’s effectiveness relative to its alternatives. (For certain conditions, such as cancer and AIDS, clinical trials often compare the most accepted treatment with a new treatment.) Most manufacturers conduct studies that show the efficacy and safety of their drug or biologic relative to a placebo (inactive) agent. For devices, the FDA requires safety and effectiveness information only for high-risk devices, such as stents, that pose a significant risk of illness or injury to patients. (The FDA approves most devices for marketing in the United States based on their similarity to previously approved devices.) Finally, for new diagnostic and surgical procedures, less clinical information is available because the FDA does not review their safety and effectiveness.

Once the FDA approves a drug, few manufacturers initiate further studies that examine its: (1) long-term safety, (2) effectiveness in patients not included in the approval clinical trials, or (3) effectiveness relative to its alternatives.

Patients have some information about differences among health care providers and the prices they charge but often they have little or no information about how well different treatments work. CMS and some private payers post information about the quality of care certain providers furnish but disseminate little information to consumers on the effectiveness of alternative medical services.

As copayments and deductibles rise, patients may become more value conscious and their demand for comparative information may increase. For example, Kolata (2006) reported that few
patients are choosing to undergo lung-volume-reduction surgery partly because of information from an NIH-sponsored comparativeness effectiveness study that compared the surgery’s clinical benefits and costs to medical treatment.

**Functions and activities of a comparative-effectiveness entity**

Whether the entity is new or an existing group, it will need to conduct and sponsor comparative-effectiveness research. Comparative research involves synthesizing existing data and research from the scientific literature. Another option is to design studies that use administrative claims data from payers. Electronic medical records might become a source of important data for comparative-effectiveness research if providers widely adopt information technology. When existing data sources do not provide sufficient information on comparative effectiveness, the entity will need to sponsor clinical trials to generate the data needed to assess comparative effectiveness.

The entity will need in-house staff with experience in designing and conducting comparative-effectiveness research. To not duplicate expertise, the entity could contract out research to public agencies and research groups with experience in conducting comparative-effectiveness research and communicating the information, such as AHRQ and its evidence-based practice centers.

The organization should be aware of the comparative-effectiveness research done by other organizations such as AHRQ, CMS, NIH, and the VA. Coordination with public and private groups would ensure that agencies do not duplicate research.

Key process issues that the entity will need to address include:

- **Identifying research priorities:** To carry out its activities effectively, the entity needs to develop a clear rationale for selecting the services to study. For the entity’s research to be relevant, its users—patients, providers, and public and private payers—should help inform the agenda.
• Producing unbiased information: Some clinical and cost-effectiveness studies show biases of investigators and their sponsors. Ensuring that analysts work objectively will be a critical issue. Ethics rules would help ensure that analysts working on behalf of the entity avoid involvement in any real or apparent conflict of interest. Ethics rules would address issues such as whether analysts can accept compensation from outside sources and requirements for regularly reporting financial interests.

• Ensuring transparency and stakeholder input: The entity’s process and methods for conducting research should be publicly documented and available to all stakeholders. Throughout the process, the entity should provide opportunities for all stakeholders to review and comment on the research methods and findings.

• Reexamining a service’s effectiveness over time: Reasons for a service’s revaluation include its use in populations not examined by the original study, new information about the service’s clinical effectiveness, and a change in practice patterns that affects the use or cost of the service.

• Disseminating information to all users: Circulating the findings from the comparative-effectiveness research to multiple audiences of different levels of sophistication, in culturally appropriate and consumer-friendly ways is a key task and should not be isolated from the review process. Rather, the entity needs to view dissemination as a crucial component of producing comparative-effectiveness research. Otherwise, the findings may not reach all potential users.

• Developing human capital: An adequate supply of qualified researchers will be needed to conduct comparative research. The entity could develop programs that train investigators and institutions to do the research.
Structuring an entity to examine and report on comparative effectiveness

The Commission has begun to explore the pros and cons of different ways to configure and finance the entity that produces comparative-effectiveness information. At this point in our deliberations, the Commission prefers a public–private option, reflecting the benefit of comparative-effectiveness information to the government, private payers, and their patients. An independent board of experts should help develop the research agenda and ensure that the research is objective and methodologically rigorous. A public–private entity might address some stakeholders’ concerns about too much federal government involvement but still provide for strong public sector involvement and oversight. A public–private entity might provide a better balance of different perspectives than an entity that is either all public or all private.

In evaluating the different governance and funding options, policymakers might consider whether: (1) users will judge the research as being objective, credible, and produced with minimal or no conflict of interest and bias; (2) the entity is independent of various stakeholders and political pressures; and (3) the entity is stable.

Different organizational options for a comparative-effectiveness entity include:

• Expanding the role of an existing federal agency, such as AHRQ, NIH, or CMS;
• Establishing a new federal agency either within or outside of the executive branch;
• Establishing a new public–private entity; or
• Establishing a new private sector entity.

Some are concerned about creating a new federal bureaucracy. Others are concerned that payers will ultimately use the information to ration health care and that it puts payers in the position of directing medical decisions. Providers and patients may not view the research as being sufficiently objective if a payer, such as CMS, houses the entity. Either a new or an existing executive branch agency may not be independent enough to take on difficult research questions and disseminate unpopular research findings. Another disadvantage of expanding the scope of an
existing federal agency is that certain stakeholders who do not support conducting comparative-effectiveness research could place funding for all its functions at risk.

A public–private entity with an external board is another option to consider. For example, the Federal Reserve System, the central bank of the United States, has a unique structure that enables it to operate independently within government but not independent of government. Although the Federal Reserve is required to report to the Congress on its activities, neither the president nor the Congress approves its decisions. The Federal Reserve consists of a federal agency (the Board of Governors) and private entities (12 federally chartered corporations known as Federal Reserve Banks). Unlike most other federal commissions, the Federal Reserve is a self-financing entity; it does not receive congressional appropriations.

Other examples of public–private entities include federally funded research and development centers (FFRDCs) and congressionally chartered nonprofit organizations. The 37 existing FFRDCs are organizations that an executive branch agency sponsors but an academic or private organization operates and that can perform work for organizations other than the sponsoring agency. By contrast, congressionally chartered nonprofit organizations do not have a “parent” agency and can receive more funding from the private sector.

Another option is to establish a comparative-effectiveness entity within a private sector entity—for example, a new or existing independent nonprofit group could take the lead generating comparative-effectiveness information. A private sector entity would minimize concerns about the government’s influence on the research agenda and the entity’s findings. On the other hand, it would be difficult for the federal government to fund such an entity without being involved in its governance. Some stakeholders who are already uneasy about the influence of manufacturers on clinical trials and reviews might be concerned about the potential for bias if a private sector group took the lead to generate comparative-effectiveness information.
Under any option, the Congress could establish an external board composed of independent experts to advise the entity about research priorities and to provide oversight for conducting research might promote transparency and the credibility of the findings.

In addition to the governance, the independence and stability of the entity will also depend on its funding. For example, an entity that relies on federal appropriations might be more susceptible to year-to-year fluctuations than an entity with mandatory funding (e.g., from the Medicare trust fund). Each year, the Congress considers the spending for services financed from appropriations. By contrast, the statute guarantees spending for services financed from mandatory sources, although the Congress has the ability to change even mandatory funding.

Voluntary contributions from private groups—such as private payers and manufacturers of drugs, biologics, and medical devices—could also be vulnerable to budget uncertainties. Private sponsors might decide to withhold or withdraw funding for any number of reasons, such as disagreeing with the selection of a service for consideration. The influence of private groups that directly fund the research on a study’s design and findings could be a concern.

Some combination of mandatory public and private funding might be a more stable source of financing for an entity that is likely to be under pressure from stakeholders. Possible sources of mandatory funding include:

- Drawing funds from the Medicare trust fund (which is financed primarily by payroll taxes);
- Drawing funds from general tax revenues;
- Imposing a tax on the nation’s annual outlays for health care services (as suggested by Uwe Reinhardt (2004)); and
- Imposing a dedicated tax on products that threaten human health, such as tobacco, products with trans fats, and alcohol.
Conclusion

Little objective, credible, and high-quality information is publicly available that compares the effectiveness of health care services furnished to patients. Comparative-effectiveness research is costly to generate and private sponsors have difficulty recouping the costs of producing the research because other users will not pay to use the research once it is publicly available. There is no federal entity whose sole mission is to conduct and disseminate comparative-effectiveness research. Consequently, the Commission recommends that the Congress should charge an independent entity to sponsor credible research on comparative effectiveness of health care services and disseminate this information to patients, providers, and payers. Use of such information has the potential to increase the value of health care spending and might reduce spending if, among clinically comparable services, less costly services replace more costly services.