Federal Initiatives To Support Rapid Learning About New Technologies

The federal government is in a unique position to generate information essential to rapid learning in health care.

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ABSTRACT: Health policy and financing reforms place increasing emphasis on the ability of doctors and patients to make informed, cost-conscious care decisions. The federal government is supporting new initiatives in Medicare to increase the supply of reliable information on the benefits and risks of health care technologies. Medicare also is working with the Agency for Healthcare Research and Quality (AHRQ) to evaluate the comparative effectiveness of prescription drugs and other items or services. The value of these efforts will depend on coordination among individuals and institutions in the public and private sectors; clarity about focus, purpose, and priorities; and adequate and reliable long-term funding. [Health Affairs 26, no. 2 (2007): w140–w149 (published online 26 January 2007; 10.1377/hlthaff.26.2.w140)]

Many currently popular approaches to improving quality, reducing cost, and expanding access to care depend on doctors' and patients' ability to make informed, cost-conscious decisions about their health care. This popularity evolved in large part from the expanding body of evidence demonstrating inefficient and inconsistent use of health care services and unsustainable trends in health care spending. If patients, clinicians, and other decisionmakers are responsible for making critical health care decisions, they need access to reliable information on the quality, outcomes, and costs of care. Despite considerable progress in producing this kind of information over the past several decades, much more is required to meet these decisionmakers' needs. This paper addresses federal initiatives, particularly those pursued by the Centers for Medicare and Medicaid Services (CMS), designed to support the efficient development of information about the benefits, risks, and costs of health care technologies.

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Although many federal programs fit within this broad scope, we focus on recent efforts that feature several methodological approaches to learning about the outcomes of health care interventions after they have obtained regulatory approval. The CMS aims to support many different analytic needs by developing an integrated database that will include linked information from claims submitted to the CMS by physicians, hospitals, prescription drug plans (PDPs), and other Medicare providers. The Effective Health Care program, under the direction of the Agency for Healthcare Research and Quality (AHRQ), uses systematic literature reviews and analysis of routinely collected clinical and administrative data, primarily from organizations with electronic medical record (EMR) systems. The CMS also supports high-quality effectiveness studies by linking Medicare reimbursement to required protocol-driven collection of prospective clinical data.

This paper emphasizes programs that involve a major role for the CMS, but many important initiatives are also under way at other federal health programs. In the private sector, health plans and other for-profit and nonprofit entities are also pursuing initiatives, in some cases offering valuable lessons that inform the federal initiatives. The objectives, progress, and limitations of these initiatives offer insight into how the federal government can support the development of better evidence for health care decision making and what will be necessary to ensure that these programs achieve their goals. Although it is clear that these efforts depend on the data, resources, and efforts of the CMS, AHRQ, and other federal agencies, the programs’ ultimate value will be determined by the degree of engagement by many stakeholders, including researchers, product developers, health plans, employers, medical professionals, and patient organizations.

The CMS’s Integrated Data Strategy

On 1 January 2006 the CMS launched its largest expansion of the Medicare program since its inception in 1965, Medicare Part D, adding outpatient prescription drugs to its list of benefits. This coverage change expanded access to prescription drugs for many Medicare beneficiaries and increased the federal government’s role as a payer of pharmacotherapy from 2 percent in 2005 to an expected 28 percent in 2006.1 In addition, the drug benefit provides the CMS, researchers, and the public with a clearer picture of the overall health care experience of the U.S. elderly and disabled populations and with an infusion of new information on the use, safety, and, potentially, effectiveness of medications in these populations.

Medicare’s drug data strategy. On 11 May 2005 the CMS issued a White Paper on its new drug data strategy.2 It outlined the CMS’s intention to integrate new drug claims data from Medicare Part D with the medical data from Medicare Parts A and B. The stated goal of doing so is to support the programmatic missions of both the CMS and the Food and Drug Administration (FDA) by supporting the FDA’s postmarketing surveillance activities and the CMS’s goal of providing evidence on drugs and drug use for a broad range of conditions. These conditions include off-
“The CMS’s goal to integrate electronic health information for all beneficiaries will require the cooperation of plans and providers.”

label uses and evidence of drugs’ effectiveness and safety in specific subpopulations, such as the elderly and those with multiple chronic conditions.

This initiative signaled the federal government’s intent for Medicare data, and other data, to play a more prominent role in the design of new policies and programs of the CMS and its sister agencies. Researchers have already identified the availability of new Medicare Part D data and their integration with existing medical data on the Medicare population as a potential panacea for some of the most pressing problems facing health policy. The high-profile withdrawal of Vioxx from the market, potential problems with other COX-2 inhibitors, and the recall of some models of implantable cardioverter-defibrillators (ICDs) have raised some concerns about the FDA’s current capacity to proactively monitor the safety of medical products used in the United States. Beyond these postmarketing safety questions, a linked data set reflecting use of inpatient, outpatient, nursing home, prescription drug, and other services and medical supplies would provide many important insights into the quality, safety, effectiveness, efficiency, and cost-effectiveness of beneficiaries’ care.

Obstacles to the initiative. The CMS and the scientific community face several obstacles in moving the Medicare integrated data initiative forward. Logistical issues include the sheer mechanical challenge of integrating vast amounts of drug claims data with other Medicare claims files and compiling those data into longitudinally analyzable files. The CMS’s goal to integrate electronic health information for all Medicare beneficiaries will also require the cooperation of the private health plans and providers that hold this information. Business confidentiality issues also could prove problematic, as the integrated data may be sufficient to reveal the underlying cost structure of a PDP or Medicare Advantage (MA) plan. Finally, the CMS will have to address privacy and confidentiality issues, such as the nature of institutional review board (IRB) oversight of the research that the CMS and others plan to conduct using these data.

A number of methodological issues will arise as well. For instance, one of the major objectives of data analysis is to focus on drug usage and monitoring rare adverse events. Ensuring against false-positive findings in this context will require a great deal of effort. Such findings are a natural by-product of multiple analyses; in a drug surveillance system that will rely on vast groups of individual researchers, the number of analyses and hypotheses will be very great, and the number of false-positive findings will be as well. Using claims data to understand causal or associative relationships will remain a challenge, regardless of the richness of the data source. There will continue to be debate about whether the information captured on either a Medicare claim or an electronic record accurately reflects the experi-
ence of the patient. One sensible initial approach to both of these concerns is to establish robust and uniform standards for data definitions, analyses, and distinct analytic subsets of the claims data, so that findings obtained through analyses of one data set could be validated in a separate data set. This approach would make it easier for the private sector, academe, and the regulatory bodies to share a common understanding regarding the meaning of claims data analyses.

- **Ensuring optimal uses of the data.** The CMS is working with AHRQ on several studies to explore the optimal uses of Medicare claims data and other observational data. Researchers are attempting to develop frameworks and algorithms to use existing and future Medicare data—particularly Medicare Part D claims—to explore drug safety issues and comparative effectiveness research. Researchers also are conducting studies to validate the accuracy of administrative data using medical records. These studies address some of the limitations of claims data and should lead to more effective use of Medicare prescription drug data. Methods and standards for cost-effectiveness analysis developed by AHRQ and others will also be increasingly important in evaluating the comparative effectiveness of drugs and other health care interventions, particularly as patients and clinicians become more accountable for the outcomes and costs of their care. On 18 October 2006, the CMS published a proposed rule on use of Medicare outpatient prescription drug benefit (Part D) claims data for research, analysis, reporting, and public health functions by the Department of Health and Human Services, federal oversight agencies, and other researchers.

**AHRQ’s Effective Health Care Program**

To learn more about prescription drugs and other health care services, in October 2005 AHRQ formally unveiled the Effective Health Care program, authorized by Section 1013 of the Medicare Prescription Drug, Improvement, and Modernization Act (MMA) of 2003. In MMA, Congress mandated that AHRQ conduct comparative effectiveness research focused on the needs of the Medicare, Medicaid, and State Children’s Health Insurance Program (SCHIP) populations. According to MMA, comparative effectiveness research should address the outcomes and effectiveness of health care items and services (including prescription drugs) and gaps in clinical research.

AHRQ responded to the need for evidence to make informed decisions by developing a research program to increase such knowledge. The Effective Health Care program (1) synthesizes knowledge in comparative effectiveness reports; (2) generates knowledge through rapid-turnaround research using deidentified data on patients; and (3) translates knowledge into targeted products for health care decisionmakers. AHRQ developed a parallel research infrastructure to fulfill these aims. The program uses existing Evidence-based Practice Centers (EPCs) and created the new Developing Evidence to Inform Decisions about Effectiveness (DEcIDE) network and the John Eisenberg Clinical Decisions and Communic-
“The DEcIDE network advances the development of scientific methods to refine new methods to analyze data.”

For the Effective Health Care program, EPCs conduct comparative effectiveness reviews that highlight what is known about health care technologies while identifying research gaps where more information is needed and unanswered questions that future research should address. The DEcIDE network, comprising thirteen research centers, fulfills item 2 above using Medicare’s claims data; EHR data; pharmacy records; private health insurance data; and disease, procedure, or device registries. In addition, the DEcIDE network advances the development of scientific methods to synthesize research and refine new methods to analyze data. With adequate resources, this network could ultimately contribute much new knowledge through prospective studies of important questions identified by systematic reviews and observational studies.

**Collaborative process.** AHRQ’s approach to implementing the Effective Health Care program involves stakeholders collaborating in a variety of ways. Experts and stakeholders recommend topics for research, which are reviewed and prioritized by an interagency steering committee with representatives from AHRQ, the CMS, the FDA, and the U.S. Department of Health and Human Services (HHS) Office of the Secretary. AHRQ also established a Stakeholder Group for the Effective Health Care program, which provides input on evidence gaps, development of research questions, and methodological issues related to the program. The program also offers opportunities for input on the development of future research topics, key questions, and draft reports by allowing the public to submit comments to AHRQ. However, AHRQ has faced criticism for not disclosing how the program incorporates these comments into its decision making.

As of July 2006, three comparative effectiveness reviews have been released: management strategies for gastroesophageal reflux disease (GERD), noninvasive diagnostic tests for evaluating breast abnormalities, and epoetin and darbepoetin for managing anemia in patients undergoing cancer treatment. The report on noninvasive diagnostic tests highlighted the limited amount of good evidence to support the use of diagnostic imaging as an alternative to biopsy in the evaluation of breast abnormalities, prompting a strong negative reaction from the imaging community. Because these findings challenge conventional clinical thinking, this report could serve to promote shared understanding among clinicians, product developers, and research funding organizations about the critical gaps in evidence and, ideally, lead to the design and execution of studies to address those gaps.

**Challenges ahead.** Although AHRQ’s Effective Health Care program provides an important opportunity to learn more about the effectiveness of health care technologies, many challenges lie ahead. For the program to be a success, AHRQ
must (1) identify research gaps on comparative effectiveness; (2) develop a research agenda that addresses these gaps, including prospective clinical trials; (3) translate research findings into information that decisionmakers can act upon; and (4) balance the need for adequate stakeholder input with the importance of producing timely and objective findings.12 These challenges are common to all organizations engaged in evaluating the comparative effectiveness of medical technologies, and past efforts highlight the importance of robust methods, adequate funding, and political support.

**Linking Medicare Payment To Prospective Data Collection**

The federal government also supports the development of new information about the benefits and risks of health care services by linking payment for services to the collection of prospective clinical, demographic, and economic data. Medicare has taken this approach on a range of mechanisms and recently on the generation of high-quality data about specific new and emerging medical technologies.

- **Early clinical trials policy.** An early manifestation of Medicare policies supporting the conduct of clinical trials was the 2000 national coverage decision on Medicare payment for the routine costs of clinical trials.13 Although the policy was designed primarily to support Phase III clinical trials of anticancer drugs, it was written to accommodate a wide range of drug and device trials. No formal evaluation of this policy has been conducted, but anecdotal reports indicate that some trials have proceeded more rapidly because of the reassurance that the costs of routine care will be covered among participating institutions. Other anecdotes have suggested that ambiguities in the policy have hindered its implementation. In July 2006 the CMS announced that it is reconsidering its clinical trials policy and seeks to expand the policy to include other types of clinical research.14

- **Coverage for specific technologies.** The first example of Medicare coverage for a specific technology linked to beneficiaries’ enrollment in a specific clinical trial occurred in 1995 through a national coverage decision (NCD) on lung volume reduction surgery (LVRS), a surgical treatment for severe emphysema.15 The CMS and the National Heart, Lung, and Blood Institute (NHLBI) agreed to collaborate on the National Emphysema Treatment Trial (NETT), a randomized, controlled trial that ran for more than five years, enrolled more than 1,000 patients, and cost more than $100 million. Final study results demonstrated that quality of life improved for some patients, while a small subset of patients experienced increased survival from surgical intervention. As a result of the NETT, clinicians gained an understanding of the negative outcomes associated with the surgery; fewer than 500 procedures have been performed, possibly sparing many Medicare beneficiaries from harm, while also saving the program sizable unnecessary expenditures.16

Medicare coverage of LVRS offered a model by which to cover certain high-impact, promising technologies while they undergo further prospective evaluation. Since that time, the CMS has issued policies linking prospective data collec-
tion to coverage for the use of fluorodeoxyglucose positron-emission tomography (FDG-PET) for suspected dementia and for diagnostic use in oncology, for the use of ICDs in patients at high risk of sudden cardiac death, and for certain off-label uses of drugs approved for colorectal cancer.17

- **Coverage with evidence development.** More recently, Medicare has taken steps to formalize its coverage policy approach under coverage with evidence development (CED), which is explained in a draft guidance document from April 2005.18 The guidance document provides a description of Medicare’s efforts to support the rapid development of high-quality evidence through coverage policy. Numerous public comments on the draft guidance were submitted to the CMS, and a revised guidance document was released in July 2006. It defines two forms of CED: coverage with appropriateness determination (CAD) and coverage with study participation (CSP). The CAD policy will be applied to items and services that are determined reasonable and necessary for Medicare coverage, and additional data collection would ensure that beneficiaries receiving the item or service meet criteria specified in the NCD. Under the CSP policy, inadequate evidence exists to conclude that an item or service is reasonable and necessary for Medicare coverage, but additional clinical research data would help clarify the benefit to Medicare beneficiaries, and then the item or service could be covered.19

 Medicare’s initial experiences with CED are already beginning to produce a large body of data reflecting the clinical experiences of patients using recently developed technologies. Certain refinements to this policy are already under way, including an effort for more explicit and systematic selection of study topics. Furthermore, future CED will be increasingly attentive to the ability of the chosen study designs to answer the most important clinical questions. It is unclear whether the ICD or PET oncology registries will provide the breadth of reliable information on the effectiveness of these technologies for making refinements in coverage policy for specific patient subpopulations. But even these examples demonstrate the potential for a constructive collaboration between multiple stakeholders to engage in the development of better evidence relevant to the care of Medicare beneficiaries. Improving the scientific value and operational simplicity of these early CED efforts will depend on the active participation of private-sector experts and stakeholders, particularly patients and medical professionals.20

- **Other initiatives.** Since Medicare’s work in CED began, several other entities have launched initiatives that are likely to assist in further refining the approach within Medicare and perhaps help increase the engagement of other stakeholders. The Institute of Medicine (IOM) has convened a Roundtable on Evidence-Based Medicine to discuss a broad portfolio of issues related to rapid creation of evidence and policies.
for decisionmakers. A recent paper has proposed the establishment of a large national program to support comparative effective reviews and clinical trials to provide payers with better information for coverage and payment decisions. In addition, a new private-sector initiative, the Center for Medical Technology Policy, funded by the California HealthCare Foundation and the Blue Shield of California Foundation, is bringing together public- and private-sector stakeholders to select and design prospective studies of new and emerging health care technologies.

Other Federal Initiatives That Support Rapid Learning

- **Veterans Affairs.** The Department of Veterans Affairs (VA) has focused for many years on the rapid and efficient development of high-quality evidence about health care technologies. The VA system is much like the government-sponsored, organized health systems in other countries. Its leaders have recognized the importance of an adequately supported applied research capacity to the effective and efficient use of their resources. There has been much progress at the VA in developing and using EMRs and quality improvement methods, and the quality and efficiency of care have improved dramatically.

- **Office of the National Coordinator.** The Office of the National Coordinator of Health Information Technology (ONCHIT) and the National Health Information Infrastructure will be critical to rapid learning. The federal government plays a vital role in setting standards, thereby supporting the development of vigorous, market-based products. Stakeholders should pay greater attention to the capacity of standard systems to gather information that is useful in measuring and reporting on quality of care and in supporting clinical research applications. In the future, EMR systems, in tandem with Medicare's integrated data strategy, have the opportunity to create valuable information that the federal government can use to make decisions about the allocation of resources to address critical research gaps and prudently purchase health care services.

- **National Institutes of Health.** The NIH focus on translational research and the clinical research Roadmap initiative are also potentially important in rapid learning. It is important and useful for the NIH to be supporting the development of infrastructure within the health care system to conduct prospective pragmatic and effectiveness studies rapidly and efficiently.

Concluding Comments

The federal government has undertaken a number of initiatives with the objective to produce reliable information, rapidly and efficiently, about the benefits, risks, and costs of alternative health care interventions. Some of these approaches boast a longer record by which to evaluate their likely contributions, while others offer only limited data by which to assess impact. Each initiative illustrates the unique potential of the federal government to make meaningful progress toward these goals, and each also offers insights into the challenges and opportunities.
The foremost requirement is recognition that the federal government is in a unique position to generate large amounts of information essential to improving the appropriateness of decisions and the quality of care. Furthermore, the use of public dollars to finance public health care programs places a unique responsibility on federal policymakers to take the steps necessary to rapidly learn and improve. For this to become reality, several requirements must be met. First, these efforts need to be better coordinated and systematically directed at high-priority issues and questions. Second, there will need to be ongoing and meaningful engagement with private-sector experts and stakeholders. Third, it will be important to recognize the applied nature of the research agenda and that decision-makers’ information needs should be a major factor in research priorities and study design, even if these needs might not be the most interesting questions to investigate from an academic standpoint. Finally, these programs will require sufficient attention and resources to achieve their considerable potential. Adequate and sustained funding, clear messages about the importance of these programs, and dedicated staff with these efforts as their primary assignment (protected from the pressures of vested interests) will be required.

Each of the initiatives described above has great potential, but their actual contributions to improved quality and more efficient care delivery will depend on stronger coordination among individuals and institutions in the public and private sectors; more clarity about focus, purpose, and priorities; better staffing; and additional dedicated and reliable funding. The private sector can also contribute greatly on its own to rapid learning in health care; however, no private-sector entities have the size, visibility, and resources potentially available to the CMS and other federal health programs.

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NOTES


7. Ibid.


12. As of November 2006, the Effective Health Care program had not undertaken any prospective comparative effectiveness clinical trials.


