Introduction: Medicare Section 1013 and AHRQ's Effective Health Care Program

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ABSTRACT

OBJECTIVE: To introduce Section 1013 of the Medicare Prescription Drug, Improvement, and Modernization Act (MMA) of 2003 and the Agency for Healthcare Research and Quality's (AHRQ) Effective Health Care Program.

BACKGROUND: AHRQ, under Section 1013 of the MMA of 2003, has been charged with conducting specific health care outcomes studies through the Effective Health Care Program. This research is aimed specifically at determining the safety and effectiveness of certain pharmaceuticals since comparative data is currently lacking. Highly utilized, high-cost (or both) treatments are the focus of the studies that will be conducted through AHRQ's Evidence-based Practice Centers (EPCs) and the Developing Evidence to Inform Decisions about Effectiveness (DEcIDE) Network. Current and completed projects are noted, including more detailed information on the reviews pertaining to treatment of rheumatoid arthritis with nonbiologic disease-modifying antirheumatic drugs (DMARDs) and biologics (anti-tumor necrosis factor [TNF]-α therapies).

SUMMARY: AHRQ's EPCs and the DEcIDE Network are studying safety and the comparative effectiveness of a number of different pharmaceutical-related topics, including the safety and effectiveness of biologic and nonbiologic DMARDs (e.g., TNF antagonists). The final reports, once complete, will be translated (explained in terms that can be more easily understood by all decision makers) and then disseminated to all stakeholders.

KEYWORDS: AHRQ, Cost-effectiveness, Medicare, TNF-α therapies (anti-TNF- α), Effective health care, Health outcomes

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On December 8, 2003, President George W. Bush signed the Medicare Prescription Drug, Improvement, and Modernization Act (MMA) of 2003. This legislation was enacted to address several shortcomings in the existing Medicare program, including the lack of a prescription drug benefit for seniors in the United States. Other important components, aside from outpatient prescription medications for seniors, are:

- grants for physicians to implement electronic prescribing
- payment/reimbursement for inhalation drugs
- recognition of nurse practitioners as attending physicians to serve hospice patients
- coverage of an initial preventive physical examination
- study/report on concierge care and coverage of chiropractic service
- Medicare Advantage quality-improvement programs and research strategies for the chronically ill
- study and report on drug importation

One of the provisions of the MMA that is relevant to health care practitioners, managed care payers, and patients, is Section 1013.

Section 1013 is titled "Research on Outcomes of Health Care Items and Services." It is significant because it focuses on the effectiveness, quality, and efficiency of delivered health care and improved health outcomes. Specifically, MMA Section 1013 is designed to address the lack of comparative data with respect to prescription drugs. This Act instructs the Secretary of the Department of Health & Human Services (DHHS), acting through the Agency for Healthcare Research and Quality (AHRQ), to conduct and support research with a focus on outcomes, comparative clinical effectiveness, and appropriateness of devices, pharmaceuticals, and services to meet priorities and requests for scientific evidence in a number of topic areas.

AHRQ is the government agency whose mission is to improve the quality, safety, efficiency, and effectiveness of health care for all Americans. The AHRQ Effective Health Care Program has 3 basic components to gauge comparative effectiveness of different treatments and clinical practices:

1. to review and synthesize existing knowledge through Evidence-based Practice Centers (EPCs),
2. to promote and generate new knowledge through the DEcIDE (Developing Evidence to Inform Decisions about Effectiveness) Research Network, and
3. to compile the findings from the EPCs and DEcIDE Network and then translate (synthesize scientific evidence about effectiveness and explain it in terms that can be more easily understood by all decision makers) and disseminate that knowledge. The John M. Eisenberg Clinical Decisions and Communications Science Center was chosen to compile the research results into a variety of useful formats for stakeholders.
This center is a collaboration of the Oregon Health and Science University, the Portland Veterans Affairs Medical Center, and the Kaiser Permanente Center for Health Research. More detailed information on the AHRQ Effective Health Care Program includes the review, evaluation, and synthesis of existing research, including potential clinical trial information, as well as other published information. AHRQ will review the already published literature and compare different therapies used to treat the same diseases. Additionally, AHRQ will identify gaps in the existing research and support this with new research. The AHRQ EPCs are centers throughout the United States with the responsibility to systematically review published and unpublished scientific evidence. The DEcIDE Research Network will then facilitate the development of new scientific evidence and analytical tools through work with public and private sectors and provide the results in an accelerated and practical format.

The information, once translated and disseminated, has the potential to be far reaching. The information will be disseminated and translated for individual practicing physicians, other health care providers, health plans, pharmacy benefit management (PBM) companies, and consumers, in terms understandable to each of them, to aid in health care decision making.

Section 1013 is designed to assist the Centers for Medicare & Medicaid Services (CMS) in determining the most effective therapies for a number of high-utilization disease states. Currently, the U.S. Food and Drug Administration (FDA) drug approval process only requires that a new drug be superior to placebo to gain FDA approval. Until now, no pharmaceutical company or government agency has taken the initiative to conduct this type of research—to directly compare therapies “head-to-head” to determine if one treatment is superior to another in a given disease state. Although this research and its results are not meant to be a government benchmark, the outcomes may have this result. For example, when the results of these studies are disseminated and published, they have the potential to shape how some of these pharmaceutical products may be covered, not covered, or reimbursed under Medicare, although it is not the intended goal of CMS under Section 1013 to withhold coverage of a prescription drug based on the results of the studies conducted therein.

According to MMA, the highest priorities for study may include pharmaceuticals (or other health care items and services) that impose a high cost, may be underutilized or overutilized, and may significantly improve the prevention, treatment, or cure of diseases and conditions (including chronic conditions) that may impose high direct or indirect costs on patients or society. As can be inferred, this legislation can significantly impact the use of pharmaceuticals as well as other products. In December 2004, following extensive public comment, DHHS identified a list of 10 “initial” priority conditions to be addressed in the Effective Health Care Program.

The DEcIDE Network is a new network of research centers that AHRQ created in 2005 to fulfill this requirement of the MMA, specifically, to generate new knowledge. The DEcIDE Network is conducting accelerated practical studies regarding the outcomes, comparative clinical effectiveness, safety, and appropriateness of health care items and services as charged under MMA 2003 Section 1013. The network comprises research-based health organizations with access to electronic health information databases and the capacity to conduct rapid turnaround research (less than 2 years duration).

The EPC reviews use a research methodology that systematically and critically appraises existing research to synthesize knowledge on a particular topic. A key component of the comparative effectiveness reviews (CERs) is the identification of research gaps and recommendations. A number of CERs are currently in progress. Additionally, 5 final reports have been issued to date.

This research involves disease states that are treated with outpatient oral medications as well as biologics. There are several high-cost disease states that the Effective Health Care Program will be addressing. Some of these disease states are included primarily because of their epidemiological impact. Others will be addressed because of the high-cost therapies available to treat them as well as the uncertain comparative effectiveness of these therapies. Still others will be addressed because they fall into both categories. One example of the latter is in the treatment of immune-mediated disease. In the area of immune-mediated disease, one can see that the current CER list includes rheumatoid arthritis (RA) or psoriatic arthritis (PsA). Some of the key points to be addressed in the RA CER include effectiveness and efficacy of the treatments relative to reducing RA symptoms, disease progression, functional improvement, and potential disease remission. A comparative analysis of the relative safety of multiple therapies will be evaluated including some of the older disease-modifying antirheumatic drugs (DMARDs) as well as the newer biologic agents (e.g., anti-tumor necrosis factor [TNF]-α agents).

The Effective Health Care Program’s CER on “Comparative Effectiveness of Drug Therapies for Rheumatoid Arthritis” will use existing research to address questions regarding which therapies are the most effective, have the best safety profile, and are the most cost effective.

The DEcIDE Network also currently has a number of ongoing projects. Initial research from the DEcIDE Network focuses on outcomes of prescription drug use and other interventions for which randomized controlled trials would not be feasible or timely or would raise ethical concerns that are difficult to address. Other DEcIDE Network projects may focus on electronic registries, methods for analyzing health databases, and prospective observational or interventional studies.

Academy of Managed Care Pharmacy Position

In April 2004, the Academy of Managed Care Pharmacy (AMCP) issued a press release regarding their position on the AHRQ Effective Health Care Program. The following is a partial listing of AMCP’s position statements:

1. The Effective Health Care Program Aims to Provide Evidence-Based Information, Not a Government Benchmark
2. The Effective Health Care Program’s CER on “Comparative Effectiveness of Drug Therapies for Rheumatoid Arthritis” will use existing research to address questions regarding which therapies are the most effective, have the best safety profile, and are the most cost effective.
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• Supports research on the comparative clinical and cost effectiveness of prescription drugs;
• Believes that to generate credible information that is comprehensive in scope, it is appropriate for the federal government to be charged with the primary responsibility for conducting or sponsoring comparative effectiveness research on prescription drugs;
• Believes that only the federal government has the financial resources to support large-scale independent studies; the expertise to provide oversight to ensure the quality and integrity of the research process; and the ability to widely disseminate the research data to all interested audiences which, when completed, will be available in the public domain;
• Believes that AHRQ, as well as the National Institutes of Health (NIH), are the logical agencies to oversee comparative clinical research efforts on behalf of the federal government since conducting research and studies is part of the mission of AHRQ and NIH, and they have the scientific and management expertise to fulfill this responsibility;
• Believes that encouraging this research will help to assure positive patient outcomes through appropriate medication use and promote the prudent management of financial resources within the health care system;
• Realizes that only limited authoritative research exists that distinguishes the effectiveness and safety profiles offered by any particular drug compared with other drugs in the same or similar treatment class;
• Realizes that those entities that pay for prescription drug benefits require this type of information so that a benefit can be designed that ensures that patients receive the best value for the resources expended.

Focus on Anti-Tumor Necrosis Factor-α Biologics

Comparative Effectiveness Review

There continues to be uncertainty about optimal drug treatments for RA, including which drug to start, when and how to combine drugs, and risks and benefits in different subgroups of patients. The purpose of this CER, “Comparative Effectiveness of Drug Therapies for Rheumatoid or Psoriatic Arthritis,” is to compare the benefits and safety of RA and PsA drug therapies. This CER will focus on the comparative benefits and safety of DMARDs, corticosteroids, and various combinations of those agents compared with each other, placebo, or other comparators (such as non-steroidal anti-inflammatory drugs [NSAIDs]). Because NSAIDs do not have disease-modifying properties, they are excluded as primary drugs from this review. A prior CER compared the effectiveness and safety of analgesics for the treatment of osteoarthritis. This new CER, “Comparative Effectiveness of Drug Therapies for Rheumatoid or Psoriatic Arthritis” will build on the results of a recent AHRQ-sponsored technology assessment on biologic DMARDs for RA, “Design and Analysis of the Cost-effectiveness of Etanercept, Adalimumab and Anakinra in Comparison to Infliximab in the Treatment of Patients with RA in the Medicare Program.” Both the older synthetic DMARDs (e.g., azathioprine, cyclosporine, gold [IM, intramuscular], hydroxychloroquine, leflunomide, methotrexate [MTX], minocycline, sulfasalazine) and the newer biologic DMARDs such as etanercept, infliximab, adalimumab, and anakinra will be included. Some of the key questions to be addressed in this research include whether the available therapies
• are able to reduce symptoms or prevent progression of radiographic joint damage,
• differ in the ability to improve functional capacity or quality of life,
• differ in their effect on maintaining remission, and
• differ in safety, tolerability, adherence, or adverse effects.

Also to be included are the comparative benefits and safety of drug therapies for inflammatory arthritis in subgroups of patients based on disease stage, history of prior therapy, demographics, concomitant therapies, and/or comorbidities.

The DEcIDE Project

The current DEcIDE project pertaining to the use of anti-TNFs for RA treatment is titled “Assessment of Factors Modulating Treatment Outcomes of Rheumatoid Arthritis.” Since RA is a lifelong disease affecting a small percentage of the general population, population-based studies are needed to assist in quantifying beneficial and adverse clinical outcomes associated with RA treatments. This project proposes a series of epidemiologic studies aimed at addressing knowledge gaps in the effectiveness and safety of specific DMARDs. The project will examine the comparative effectiveness and safety of older DMARDs versus newer biologic agents, including the anti-TNF agents. The following are also goals of this DEcIDE project:
1. To define a cohort of patients with RA among state Medicaid enrollees to determine the prevalence of use of selected DMARDs.
2. To explore several outcomes as measures of relative effectiveness of specific agents/combinations such as prevalence of cotherapy with corticosteroids, NSAIDs and narcotics, and adherence to standard regimens and continuation of use
3. To determine the incidence of congestive heart failure (CHF) in the RA cohort and test whether RA patients receiving anti-TNF-α therapy (etanercept, infliximab, and adalimumab) are at increased risk of developing CHF versus patients receiving traditional synthetic DMARDs
4. To determine the incidence of selected infection outcomes in the RA cohort and test whether RA patients receiving anti-TNF-α (etanercept, infliximab, and adalimumab) or anti-interleukin-1 (anakinra) are at increased risk of serious infections versus patients receiving traditional synthetic DMARDs

Summary

The MMA is not just about drugs for seniors. Section 1013 of the MMA includes the AHRQ Effective Health Care Program to identify knowledge gaps and study highly utilized, high-cost (or both)
treatments (drugs and other) for many diseases. Translation of the knowledge will occur, and dissemination of the knowledge will be reported to all stakeholders. Stakeholders include health plans, physicians, and patients/consumers. The information identified through this program, although not meant to be a “benchmark,” may well turn out to be the government standard.

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REFERENCES