Medicare's Coverage With Evidence Development: A Policy-Making Tool in Evolution

Coverage with evidence development is a tool used by the Centers for Medicare & Medicaid Services (CMS) in an attempt to bring a new rationale to payment decisions and, ultimately, cost savings to the Medicare program. Here we explore the latest evolution in Medicare coverage policy and coverage with evidence development (CED), discussing how it developed and what it means for practicing oncologists.

Origins of CED

Evidence-based medicine (EBM), making treatment decisions on the basis of previous examples of effectiveness or ineffectiveness, is the foundation for identifying gaps in evidence and for evaluating existing evidence to establish best practices.[1] With the variety of oncologic treatments, diseases, and presentations, oncology has been described as depending on evidence-based decision making, possibly more than any other medical specialty.[2] ASCO's clinical practice guidelines (CPGs), which synthesize evidence from clinical research into clear and useful applications for practicing oncologists, are a familiar example.

Quality and Pay-for-Performance

During recent years, use of EBM and adherence to such tools as CPGs has been linked to quality assurance measures, and quality assurance to payment. Although there are many benefits to using CPGs as a tool to evaluate quality, there are also drawbacks. "CPGs are viewed as credible sources of data for what might be considered effective care. However, CPGs are primarily designed as tools to broadly inform patient care, rather than a roadmap for the treatment of a specific patient," wrote Wolff and Desch in a 2005 *Journal of Oncology Practice* article.[1] "CPGs have a stated goal to improve quality of care, but there is significant variability in how quality is defined and they commonly lack validated quality indicators. In our highly fragmented health care delivery system that is geared towards the provision of acute care and technical procedures, the use of CPGs to guide reimbursement policies in the absence of robust quality indicators could in some cases create perverse incentives."

Pay-for-performance (P4P) models in oncology, which to this point have rewarded quality reporting through financial incentives, gained steam through CMS demonstration projects in 2005 and 2006. In general, the goal of P4P is to tie payments to quality and efficiency standards in an effort to get better outcomes for beneficiaries, and programs include hospitals as well as physician groups.[3] A major challenge with P4P models is developing adequate and sustainable quality measures that provide a reliable baseline against which to evaluate performance.[3] While the Medicare Modernization Act of 2003 (MMA) promoted P4P models, they are less of a priority for the current Congress.

New Terms Applicable to Medicare Coverage Policy

- Evidence-based medicine (EBM)—Medical decision making that utilizes clinical data as its basis.
- Pay for performance (P4P)—Financial incentives to promote quality of patient care by rewarding physicians for meeting quality performance measures.
- Comparative effectiveness (CE)—Evaluation of multiple treatments for the same disease or condition to determine which is the best therapy.
- Least costly alternative (LCA)—The less expensive of multiple comparable treatment options; a local coverage decision that limits reimbursement to the price of the cheapest comparable treatment.
- Coverage with evidence development (CED)—Medicare coverage of a treatment or technology conditioned on data gathering through a clinical trial or registry to determine its effectiveness.

ASCO's Quality Oncology Practice Initiative (QOPI), launched in 2002, is an oncology-specific quality-improvement program for which measures are developed and revised by practicing oncologists and measurement experts. QOPI is the result of ASCO's National Initiative on Cancer Care Quality (NICCQ),[4] which was established in response to the Institute of Medicine's 1999 report "Ensuring Quality Cancer Care."[5] Among the strengths of QOPI is its basis in clinical guidelines and established standards and its high clinical relevance.[6] The program allows participating practices to evaluate their performance alongside that of their peers based on semiannual abstracting of medical records,[7] in a real-world, essentially real-time comparison of treatment decisions in medical practice. Additional information about QOPI is available at www.asco.org/QOPI.

CMS has its own quality program, the Physician Quality Reporting Initiative (PQRI), which emphasizes "value-based purchasing," quality of care, and successful physician reporting.[8] PQRI is based on 74 quality measures and rewards reporting with bonus payments of 1.5% of allowed Medicare charges for the reporting period—July 1, 2007, through December 31, 2007. A minimum 80% reporting rate is required to be eligible for bonuses. CMS has proposed extending the PQRI into 2008. Additional information about PQRI is available at www.cms.hhs.gov/PQRI.

Comparative Effectiveness and Alternatives

Growing out of EBM is the concept of comparative effectiveness (CE), which refers to the evaluation of multiple treatments for the same disease or condition to determine which is the best therapy. Earlier this year, Representatives Tom Allen (D-ME) and Jo Ann Emerson (R-MO) introduced the Enhanced Health Care Value for All Act (H.R. 2184) to propose funding for new CE research, to empower the Agency for Healthcare Research and Quality [AHRQ] advisory board to prioritize CE research, and to more closely connect research with medical practice,[9] and, presumably coverage for medical care, The introduction of this Act was closely followed by additional calls for CE legislation from the BlueCross BlueShield Association[10] and from Senator Hillary Rodham Clinton (D-NY).[11]

Although there are indisputable benefits to identifying the most effective treatments for various ailments, the issue of effectiveness is not black and white. Who decides what "effective" means? If a drug dramatically improves a patient's quality of life but has no impact on overall survival, is more or less effective than one that prolongs survival marginally but with no quality improvement? Further, who determines comparability? If two products are similar in effectiveness but have different price tags, the less expensive one will likely be deemed the least costly alternative (LCA), essentially a local coverage decision that limits reimbursement for both products to the cost of the cheaper one.[12]

PET Registry

The National Oncologic PET Registry is a familiar example of CED. The PET Registry demonstrates both the attractive and the nuisance features of CED. Patients with cancer diagnoses not currently covered for [F^{18}]fluorodeoxyglucose positron emission tomography reimbursement under existing policy can receive reimbursement if their physician and provider participate in the registry.

On the other hand, this is a situation in which "medical necessity is decided contingent on participation in a trial or study," says Sean Tunis, MD, founder and director of the Center for Medical Technology Policy in San Francisco, CA, and former CMS chief medical officer and director of the Office of Clinical Standards and Quality. "Limiting coverage of a new technology to only patients in a trial is a significant limitation."

For additional information on the PET registry, visit www.cancerpetregistry.org.

"For every product, there is some subset of the population for whom the product is a cost-effective alternative," says Donald W. Moran, founder of health research and consulting firm The Moran Company. "If a product is superior for 80% of the population versus 20%, you can't restrict coverage to just those 80%." This raises concerns that coverage may be confined to only the *most* effective treatment option for the majority of patients, rather than *an* effective treatment option.

The Medicare Trust Fund

This context of evidence, quality, and effectiveness forms the conceptual basis for CED. There is, however, an important financial element at play as well. In 2005, *The Washington Post* reported that the Medicare Trust Fund is projected to be exhausted in 2019, a full 20 years ahead of the forecast demise of the Social Security trust fund.[13] Health care costs continue to rise, and so does the percentage of the gross domestic product (GDP) being spent on medicine. Medicare decision makers are therefore looking for ways to lower the cost of health care by increasing the value of services provided, eliminating unnecessary costs and complications, and emphasizing prevention and personalized care,[14] with the goal of allowing Medicare services to continue within the limitations of the existing system.

CED Today

Although the concepts that form its background are familiar and well established, CED is a relatively new approach to Medicare coverage, first introduced in 2005 and then refined in 2006; it "links Medicare coverage of specific promising

technologies to a requirement that the patients participate in a registry or clinical trial."[15] Ultimately, the data generated in the trial or collected through the registry is intended to be used as the basis for future coverage decisions once it is determined whether a treatment is reasonable and necessary. CED has also been referred to as a way to develop a "learning-based health care system" and the coverage to support it.[14]

Additional, secondary subtypes of CED include coverage with appropriateness determination (CAD), defined as "the function of gathering data to assure that an item or service was only being provided appropriately according to the clinical criteria specified in the coverage decision,"[15] and coverage with study participation (CSP), wherein a treatment would be considered reasonable and necessary "if the patient was enrolled in a clinical study that would ultimately provide reliable evidence of the health benefits and risks of the item or service."[15]

A stated advantage to CED is that it "provides a mechanism for promising but unproven technologies to get into practice sooner, conditioned on evidence generation, which is of benefit to both clinicians and patients if it's done right," says Sean Tunis, MD, founder and director of the Center for Medical Technology Policy in San Francisco, California, and former CMS chief medical officer and director of the Office of Clinical Standards and Quality. However, he says, "there are also risks because you can't differentiate ahead of time between promising and premature."

Perhaps that is the goal in CED: limiting payment to only promising and proven technology that will have the greatest positive impact (providing earlier access in targeted ways to promote premarket learning[14]) rather than spending limited funds on treatment modalities that may prove ineffective or even potentially harmful. But that is hard to say because the actual execution of CED is still quite vague. "CED and CE are both a Rorschach test, buzz phrases, not a description of an operational program," says Moran. "CED, until now, has been a limited number of circumstances to make coverage conditional on manufacturer testing and data. [We] don't know what they'll do with the data they'll collect. The agency can change policies to be more restrictive if the data suggest it."

According to Moran, there are two issues in practice with CED—the speed of a new product to market and its coverage, and rules for off-label usage. "It's possible that CMS will flip the No. 1 burdens to the FDA, where approval will equal coverage," he says. "They may add new criteria by defining medical necessity more narrowly than the FDA on label, and then slow the introduction of new agents." He suspects that there will be tighter restrictions on off-label uses that will slow the diffusion of products into new areas and also slow access to new agents and new uses. "The theory is, any agent for anybody at any time is a bad coverage policy, so let's do something more restrictive."

Opportunities and Challenges

Ideally, CED would open up discussion as to how manufacturers, physicians, and payers can most rapidly and efficiently improve the evidence available for decision making and locate the areas where more evidence is most needed. "Clinicians are critical participants in identifying those deficiencies: what are the most important unanswered or inadequately answered questions," Tunis says. "The key limiting factor in a more rational and efficient health care system is better evidence on what works and what doesn't and how well. Clinicians and patients are the most important source for that information."

Tunis states, "Coverage policy both by Medicare and other payers could effectively be used as a tool to generate the information that's missing. . . [One can] get stuck on the debate between the product development community and payers on whether evidence is sufficient for coverage." He believes it is critical to broach that dialogue.

Like effectiveness, though, the rules for adequacy may be a matter of judgment. But, says Moran, defining those rules may be up to the manufacturing community. "Manufacturers could go to Congress and request that they lay out a set of rules, but they may obtain due process at the price of an explicit set of rules and a mandate to do it," he says.

Moran describes the manufacturing community as being somewhat schizophrenic at the present time, as it navigates between the disadvantage of having required rules and the potential advantage of having CMS regulate coverage. His prediction is that, because CMS and manufacturers have approached restrictive coverage decisions one at a time until now, they will probably continue to do the same in the future.

Says Tunis, "[In] the notion of payers using evidence-based medicine to make coverage decisions, the premise is that telling product makers how high the bar is, all that's necessary is for them to do the studies to get the information." Whereas historically, payers have sat back and simply told manufacturers to jump, he says, they are now taking a role in helping with that leap. "They are playing a role in generating the information required to make the decisions. This is transformational in terms of recognition of a different role of payers in clinical research."

CED may be a means of collaborating toward an end, and has clearly led to the sense by the Medicare program that improving quality of information for decision making is as much their responsibility as anybody else's. That being said, it will still be difficult to figure out priorities and coordinate

Clinicians As Resources in Learning-Based Health Care

Although comparative effectiveness and evidence-based medicine are not new players in the field of medicine, as the nature of care interactions changes in response to new trends in Medicare coverage policy, the roles of patients and clinicians will evolve in tandem with that of payers.

"It would be pretty hard for individual oncologists to have a thorough and systematic understanding of the various alternatives," says Center for Medical Technology Policy Founder and Director Sean Tunis, MD. But despite the challenges that may arise as coverage with evidence development gains momentum, it creates an opportunity for physicians to interact in different ways with their patients by becoming more familiar with resources like the Foundation for Informed Medical Decision Making (www.fimfm.org) and the patient versions of clinical guidelines.

"Oncologists can become a resource for resources by using them themselves and providing them directly to their patients," Tunis says.

all of the factors that will contribute to effective CED. "It's not self-evident what the right questions are," Tunis says.

Also, CED presents a challenge in that it offers new treatments to a limited universe of patients by restricting coverage to trials and registry. It is a logical arrangement, but it presents a practical challenge. Another key challenge is determining how best to incorporate judgments of clinicians and patient preferences into coverage decision making, alongside rigorous medical studies. "While coverage decisions, particularly from Medicare, are doing better with regard to a focus on scientific evidence, they're not doing as well on how to incorporate unbiased clinical judgment and expectations of the community in coverage decisions," says Tunis. "You can go too far in application of the evidence-based medicine framework."

Evidence Gathering

Further, the issue of generating the meaningful evidence necessary to make coverage decisions is no small challenge. "Meaningful means very robust, statistically significant evidence that product A works better than product B for a specific indication," says Moran. "That's good. People will use that. But you can do all of the rigorous evaluation in the world and 80% to 90% will come out with ambiguous conclusions. The idea is that there will be enough slam-dunk, no-brainer [results] to make it worth it, and that's an empirical question. People must have a certain amount of humility about what data will come."

Another question is that of whose job it is to pay for CE studies and studies of new technologies to generate sufficient evidence. Tunis suggests the formation of a new national institute on CE with several million or a billion dollars to spend on comparative studies with the goal of generating highly robust data. "The challenge is that many believe the evidence should be gleaned from routinely collected data in health care, linking insurance databases, and so on," says Tunis, but he doesn't believe that systematic reviews will even begin to supply the kind of robust data achieved with comparative studies.

Dr Tunis referred to the practice changing Antihypertensive and Lipid-lowering Treatment to Prevent Heart Attack Trial (ALLHAT), which was an NIH-funded, randomized, double-blind, controlled hypertension treatment trial in 42,418 patients, which reported that a thiazide-type diuretic (chlorthalidone) was superior to a calcium channel blocker (amlodipine), an angiotensin-converting enzyme inhibitor (lisinopril), and an alpha1-blocker (doxazosin) in preventing the new onset of heart failure (HF).[16] "If the ALLHAT study had been an administrative database study, it probably wouldn't even have gotten published, much less generated enthusiasm," he says. "The policy dialogue hasn't really scratched the surface of [the potentially practice-changing nature of administrative database studies]. I hope the [AHRQ] Comparative Effectiveness Advisory Board will create good priorities to address that."

Before CMS or anyone else invests money in comparative effectiveness research, Tunis says, there needs to be a more clear idea of how to use that money. "It's manageable to think through those questions on a topic-specific basis, but hard to have that conversation generically. Figure out what the job is first, and then select the tool," he says. "Sufficiently designed and run prospective studies are going to contribute a lot to the missing evidence, and we're going to see much less of a role for systematic reviews, and more claims data analysis."

Tunis also points out that in future discussions sections of clinical reports, it is very seldom that investigators suggest a systematic review or a registry as the next step; it is nearly always a large, randomized controlled study. Recommending analysis of routinely collected data or systematic reviews as a means of collecting robust evidence for CED would be a disconnect—an apparent inconsistency that would need to be addressed.

"With respect to manufacturer-funded research, it is possible to see positives and negatives," says Moran. "On the one hand, we rely on manufacturer-funded research to determine

safety and efficacy; it's the FDA's role to ensure that the research that is done meets its standards, under threat of disapproval. Hence, the conflict [of interest] issue is probably manageable. On the other hand, assuming that you would make the cost of doing this stuff part of the cost of the drug approval process, you're going to materially increase the [research and development] cost load that the sector has to amortize across everything. This could be mitigated somewhat if manufacturers didn't have to incur this cost until after phase III approval, but the aggregate cost would still be there."

Considering Moran's earlier observation that most research will likely yield ambiguous results, these trials may not yield the return on investment manufacturers need to make them worthwhile. "This argues for being far more selective, concentrating research resources on areas where there is prima facie evidence of performance differentials. This approach, however, would raise important questions of who gets to decide what products get tested," says Moran. "[This] balancing act. . . is one of the most important reasons I believe that, if this stuff is ever going to get institutionalized, it should go through the legislative process, which for better or worse is the mechanism industrialized societies use to resolve issues when there is no obvious right answer."

Priority Setting

Among recommended priorities for CED are (1) identifying and prioritizing gaps in existing research and evidence, (2) standardizing methods for gathering and processing data and making decisions, (3) establishing and disseminating a technologic infrastructure to support the research, and (4) allocating or generating funding to support initiatives and studies.[17]

As Tunis pointed out, physicians and their patients will play a significant role in spurring the discussion of where deficiencies lie. Additionally, says Moran, anything physicians can do to speed the dissemination of information and existing knowledge into medical practice is a good thing, and not just in the field of oncology.

Where methodology is concerned, types of trials and their requirements, performance measures, medical necessity decisions, disease management and quality improvement programs, case management protocols, decision support tools, priority and goal setting, and strategic planning are only some of the elements that need to be considered.[18] The foundation must support the structure that will be built on it, and likewise, that structure must match its foundation. Without constant coordination and clear planning from the beginning, the potential for contradiction is great.

Once the need for research has been identified and methodologies for accomplishing evidence goals have been set, information management will be critical. *JOP* has discussed electronic health records (EHRs) extensively, and has highlighted their ability "to integrate an individual's multiple, physician-generated, electronic medical records and the patient-generated personal health record. Intended to be comprehensive, the EHR should facilitate optimal management of the health of an individual or, when used in aggregate, of a population. EHRs should allow sharing of information about patients between any authorized providers. A patient should be able to enter any health care setting, provide authorization, and then consult with a provider who has ready access to his complete health record. EHRs should be securely linked over the Internet and should be integrated seamlessly with medical information for the education of both providers and patients."[19] Utilizing technology will facilitate better application and distribution of data,[20] allowing better identification and follow-up for health care through an active surveillance infrastructure.[14]

The Future of CED

Although CED is currently nebulous in many ways, it is certain that changes will be coming in Medicare coverage policy, and that evidence-based decision making will be at the forefront. Oncology currently receives what might be considered special treatment when it comes to off-label use: "Somewhere between 50% and 80% of cancer chemotherapy involves one or more off-label uses of approved drugs, and, if third-party payers did not cover those uses, quality cancer care would suffer."[2] EBM has been the basis for this policy, and ASCO was a driving force in securing coverage for off-label uses.[2]

"Until now, chemotherapy agent policy has been more liberal than any other area," says Moran. "Even a uniform policy identical to other drugs would be more restrictive compared to now. At a minimum, the presumption is that rapid diffusion and off-label use will get more restrictive." Moran also predicts an explicit regulatory mechanism, specifically for off-label chemotherapeutic agent use.

Additionally, with CED comes the possibility for what Tunis called "decision-based evidence making," or creating the evidence to support the decision. Whether this will negate the validity of the decision or the evidence remains to be seen, but it is one potential challenge that CED will have to face as it finds its form. Another challenge is that the shift to simultaneous consumption and generation of evidence will mean more work for clinicians and patients alike, but effective methodologies and technologic infrastructure should ease the transition.

CED and EBM are already part of the clinical experience, but as the role of payers changes through CED, the role of patients will probably change as well. "It will be an increasingly common experience for patients to be enrolled in

prospective studies . . . and patients will get more accustomed to being both a patient and an evidence source," Tunis says. "The evolution will be that patients understand that part of receiving clinical care will be contributing to the information going into studies. It's not just the use of evidence, but the creation of it."

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