Medicare CED: Considerations and Implications

by Kirk L. Dobbins

Medicare beneficiaries, like many patients in the United States, often expect medical treatment using the latest medical technology. The Centers for Medicare & Medicaid Services (CMS), which administers the Medicare program, has a daunting task in meeting Medicare beneficiaries' expectations.

Under sections 1861(s) and (t) of the Social Security Act (SSA), healthcare items that may be used to treat Medicare beneficiaries range from drug and biologicals to medical devices and durable medical equipment.[1] The Medicare program cannot reimburse providers who use those items until they are covered under section 1862(a) of the SSA by CMS or a Medicare contractor as reasonable and necessary for the diagnosis or treatment of illness or injury, or to improve the functioning of a malformed body member.[2] There is no statutory or regulatory definition of reasonable and necessary in the Medicare program. CMS, however, generally has interpreted reasonable and necessary to mean that the item or service should improve the health outcomes overall for Medicare beneficiaries.[3]

In light of the rapid pace of development of new medical technology and treatment, CMS is faced with a significant challenge in developing an efficient process to determine what technology and treatments effectively improve the health outcomes of Medicare beneficiaries at a reasonable cost. In April 2005, CMS issued a somewhat controversial draft guidance on Coverage with Evidence Development (CED) to help meet that challenge.[4]

CED is not entirely new, but it is the latest iteration of CMS' policy of conditioning Medicare coverage on the further collection of clinical data or evidence to evaluate the effectiveness of certain healthcare items on the Medicare population. CMS previously issued coverage determinations conditioned on the collection of additional clinical data known as "coverage with conditions" and later determinations known as "coverage under protocol."[5]

CED relies on evidence-based medicine to expand coverage of items by Medicare through National Coverage Determinations (NCDs).[6] After a 60-day public comment period on the draft CED guidance, CMS issued a Fact Sheet in July 2005 that responded to concerns raised by approximately 65 organizations, including manufacturers who submitted timely comments on the draft guidance.[7] CMS explained in the draft CED guidance that the agency is committed to ensuring that advances in medical technology are available for Medicare beneficiaries.[8] CED will allow Medicare to pay for items under conditions that "assure significant net benefits of the treatment for beneficiaries" and to collect additional information.[9] CMS believes that evidence gathered under CED also will provide a better understanding of the risks, benefits, and costs of alternative diagnostic and treatment options.

CMS does not intend to duplicate the regulatory determinations of the Food and Drug Administration (FDA) or to assume the role of the National Institutes of Health (NIH) in sponsoring clinical trials. Rather, based on

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my discussions with one CMS official involved in the development of CED, CMS intends for CED to complement FDA's role. FDA will continue to exercise its authority to determine whether drugs, biologicals, and medical devices are safe and effective. CMS will use CED, however, to collect evidence on the long-term effectiveness of certain FDA-approved products for Medicare beneficiaries. CED will expand Medicare coverage of FDA-approved products through evidence collection that will be used to determine whether certain healthcare items improve health outcomes for Medicare beneficiaries. Expanded coverage and collection of additional evidence under CED will address situations where the effect on net health outcomes and benefits to Medicare beneficiaries is not studied or apparent for new medical technology—even after premarket approval or clearance by FDA.

Usually, CMS will require CED only when it has determined that it is unlikely that additional clinical studies will be conducted on the effectiveness of a particular medical technology on the Medicare population. CMS believes that evidence obtained through its CED initiative will help physicians and patients obtain the most beneficial treatments at the lowest possible cost. Only technology and treatments that have the potential to improve health outcomes will be considered for CED.

CMS' goal under the CED initiative is to foster systematic, protocol-driven data collection on new medical technology to determine whether the technology has the potential to improve health outcomes. In the draft CED guidance, however, that goal is balanced against the cost and burden associated with the data collection.

Statutory Authority for CED

Data collected under CED will allow CMS to expand coverage of items that have been covered in NCDs under section 1869(f)(1)(B) of the SSA.[10] CMS implemented CED under section 1862(a) of the SSA.[11] Section 1862(a)(1)(A) excludes from Medicare coverage and payment those items that are not reasonable and necessary for the diagnosis or treatment of illness or injury, or to improve the functioning of a malformed body member.[12] CMS has interpreted section 1862 of the SSA to mean, in some cases, that an item or service is reasonable and necessary only when specific data is collected to ensure that the care provided is likely to improve health outcomes.[13]

When Is CED Appropriate?

According to CMS, there are two general circumstances in which the clinical care that is provided may be considered reasonable and necessary only when accompanied by protocol-driven data collection under CED. In the first circumstance, a particular medical device or drug may have been shown to improve health outcomes in a broad population of patients, but additional evidence is needed to determine whether the device or drug is reasonable and necessary for Medicare beneficiaries. CMS would require data to be collected and reviewed by the provider at the time the item or service is delivered. The data may include patient characteristics, side effects of treatment, clinical conditions, and test results. This additional evidence, along with published scientific evidence and other available information would be used to support appropriate treatment, and Medicare coverage and payment. Data collected at the time of treatment also may be important in ensuring that a Medicare beneficiary's care is reasonable and necessary over a period of months and years. This first circumstance is particularly applicable to treatments such as implantable devices (e.g., implantable cardioverter defibrillators), provided to patients with chronic illnesses.

The second circumstance in which CED may be required is when a particular medical technology or treatment has yet to conclusively demonstrate an improvement in health outcomes, but existing information "clearly suggests the intervention may provide an important benefit."[14] CMS would require the collection of additional data to support Medicare coverage. In this circumstance, CMS may determine that sufficient evidence demonstrating improved health outcomes can be obtained only if additional data are collected, reviewed, and submitted at the time of service.

Two recent examples of the second general circumstance provide insight on how CMS will apply CED. CMS required CED for coverage of certain off-label uses of anticancer drugs.[15] Off-label uses of four drugs approved for the treatment of colorectal cancer may be covered if the patients receiving the drugs are enrolled in one of nine clinical trials sponsored by the National Cancer Institute (NCI). CMS has found a "sufficient inference of benefit" when off-label uses of these anticancer drugs are used in the context of NCI-sponsored clinical trials. The agency based its inference on the evidence of safety and effectiveness of the chemotherapy for the FDA-labeled use, the decision by NCI to conduct the trial for additional uses, and the additional patient protections provided to patients receiv-

ing protocol-driven care.[16] Based on the safeguards inherent in NCI-sponsored clinical trials, including patient evaluation, selection, and reasonable use of cancer chemotherapy, CMS concluded that coverage for off-label use of chemotherapy could provide clinical benefits to Medicare beneficiaries with cancer by influencing the management of patient care in a clinically-relevant context.

CED also was required for the coverage of Fluoro-D-Glucose Positron Emission Tomography (FDG PET).[17] Medicare initially did not cover FDG PET for cancer diagnosis, finding instead that there was insufficient evidence to conclude that FDG PET scanning was reasonable and necessary for diagnosing all cancers. Medicare coverage was expanded later with specific limitations. CMS determined that FDG PET scanning was reasonable and necessary for certain malignancies and specific clinical indications in the context of certain data collection requirements, including prospective clinical studies and clinical protocols, under CED or "coverage based on evidence of benefit."[18] The effective and accurate use of PET scans for certain cancers can be ensured only by data collection. As in the case of CED to cover off-label uses of certain anticancer drugs, CMS found that data collected in the CED for FDG PET have the potential to improve patient management and health outcomes.

CMS' decision to cover carotid artery stenting conditioned on postapproval studies is another application of CED that may be helpful in understanding the agency's reasoning when Medicare coverage is conditioned on data collection.[19] In the October 2004 coverage decision, CMS decided to cover percutaneous transluminal angioplasty with carotid stent placement in an FDA postapproval study for a number of reasons including: 1) the significant disease burden from stroke and carotid artery stenosis in the Medicare population; 2) the treatment is promising but has considerable patient risks; and 3) the data collected may "reinforce" data on health outcomes and adverse events.

Important Factors for Consideration

CMS intends to consult with external experts and stakeholders, and conduct critical analysis in developing both criteria and a process for determining when to apply CED. The initial focus will be on identification of a small group of high-priority pilot efforts on topics for which there is agreement that better evidence would be valuable in expanding access to specific technologies.

The initial pilot efforts have not been finalized. According to the draft CED guidance, however, CMS believes that data collection under CED may be valuable under a number of circumstances.

- The item or service is likely to provide benefit, but there are substantial safety concerns or potential side effects that are inadequately analyzed in current clinical literature.
- The available clinical studies may not have adequately analyzed the risks and benefits in specific subgroups or in patients with disease characteristics that excluded them from clinical trials, which also make up significant segments of the Medicare beneficiary population that would receive the treatment if covered.
- The risks and benefits of surgical procedures may not be evaluated extensively because limited information about risks and benefits has been developed for Medicare beneficiaries. For example, some noninvasive FDA-approved devices may be well characterized in terms of safety, but clinical effectiveness for certain Medicare beneficiaries is not well studied.
- When the current evidence is not generalizable to providers/facilities or the Medicare population has not been included in the available clinical studies, new evidence development may help evaluate the safety and benefit of requested items for Medicare beneficiaries.[20]

In each of these circumstances, insufficient data exist on the effectiveness, risks, and benefits for the Medicare population. When faced with these circumstances, CMS may consider coverage of a promising item or service conditioned on a protocol-driven data collection methodology or other prospective data collection, to gather information that the agency believes is sufficient to determine whether the items are reasonable and necessary for Medicare beneficiaries.

Data Collection Requirements

Although CMS does not intend data collection under CED to be overly burdensome, manufacturers and providers may not share the agency's assessment of how truly burdensome evidence collection may be. CMS intends to add to existing data, not replace or repeat existing information if it is sufficient to make a coverage determination under ordinary NCD procedures. Fortunately, CMS does not intend to require the use of a uniform design for evidence development under CED. Instead,

evidence development through data collection will vary according to the use or purpose of the healthcare item or service and the Medicare patients using them.[21]

Various study designs may be used to collect evidence in CED.[22] CMS recognizes that randomized clinical trials (RCTs) provide the best evidence of effectiveness when done properly, but also provide evidence that is not as broadly generalizable beyond patients with specific characteristics. RCTs also are very costly. Other rigorous studies that may provide valuable evidence include longitudinal or cohort studies for long-term evaluation of patient safety, and the collection of information on the course of a disease treated with a particular drug or device. Prospective comparative studies (or practical clinical trials) also can be used to collect evidence on the benefits of items. These studies can be used to evaluate a broad range of real-world outcomes such as quality of life or cost effectiveness, in addition to monitoring patient safety and health outcomes. Databases are the least costly and most informal means of evidence collection.

CMS will "encourage" study designs that are appropriate for the type of information needed; that adhere to scientific, medical, and ethical principles; and that require qualified scientific oversight, hypotheses, and data collection methods.[23] Other required parameters that will be scrutinized closely by CMS include sample size, patient safety and monitoring, training of providers and others, data security, efficiency, and data collection burden.

The draft CED guidance procedures to ensure confidentiality and proper use of patient data have been controversial. According to the draft CED guidance, CMS or the public may use the evidence collected for research. There are no uniform procedures, however, to protect the confidentiality of patient data. Instead, CMS will rely on researchers or organizations to comply with existing laws including the privacy protections under the Health Insurance Portability and Accountability Act of 1996.[24] CMS also will require the use of de-identified data for analyses.

Data collection instruments should be designed so as to minimize the burden to providers and patients, but still be able to deliver information critical to a determination of the reasonableness and necessity of the particular items. The agency also will recommend collaboration among providers, scientists, and others to develop innovative study designs.

Many concerns have been raised in comments submitted to CMS on the CED draft guidance. Some organizations fear that CED will result in decreased access to certain healthcare items. Other organizations are concerned about the lack of a clear reimbursement mechanism for the evidence collection. Several organizations have raised concerns in public comments that there is no clear indication of when or whether CMS will pay for the collection of additional data. Some organizations also are concerned that CED will limit coverage only to those Medicare beneficiaries willing or able to participate in clinical trials, registries, or other forms of evidence collection.

CMS has not yet fully responded to many of these and other concerns raised in public comments on the draft CED guidance. CMS has responded, however, that it intends to apply CED to expand coverage under NCDs. CED will be used infrequently because approximately 90% of coverage determinations are made at the local level through Local Coverage Determinations (LCDs) under section 1869(f)(2)(B) of the SSA.[25]

CED Policy May Change

CMS has provided some guidance on when CED may be required. Because the CED guidance is still in draft form, however, these parameters are subject to change based on CMS' analysis and response to public comments. This was confirmed recently in a conversation with a CMS official. CMS officials have stated publicly that a second draft guidance will be issued in the next few months. CMS will respond to additional concerns and clarify the agency's CED policy in the second draft guidance. CMS will continue to expand coverage under NCDs, but other portions of the draft guidance will be revised and, hopefully, clarified. Interested manufacturers should follow CED policy guidance closely and take advantage of future opportunities to submit public comments.

One area that needs clarification is when CED will be required for FDA-approved products. CMS has indicated that if FDA has approved a product for one indication, but has not made a determination on a different indication, CED may be "particularly helpful in providing evidence." At the same time, CMS has indicated that, for the most part, the agency or its Medicare contractors will cover devices and drugs that have obtained FDA approval, and CED is unlikely to be applied.[26] These statements appear contradictory. One reading suggests that CED may be applied to evaluate whether coverage should be expanded to an unapproved indication for an FDA-approved product while additional evidence is

collected to determine its effectiveness for the Medicare population.

Many questions and concerns have been raised by the current draft CED guidance. One fact is clear: FDA approval or clearance is not a guarantee of Medicare coverage. At a minimum, CMS or its contractors must determine that FDA-approved drugs and devices are reasonable and necessary. If clinical trial data, or scientific or medical literature, fail to provide information sufficient for CMS to make its coverage determination, the agency may require CED as a condition of Medicare coverage and payment. If possible, manufacturers should consider developing evidence through clinical trials, reviews of published scientific literature, or other means as early as possible to demonstrate not only the safety and effectiveness of their products for the Medicare population, but also that these new products may improve health outcomes for the Medicare population. Manufacturers also should become familiar with Medicare coverage requirements and procedures for NCDs and LCDs, and related guidance.

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[1] 42 U.S.C. § 1395x(s), (t).

[2] *Id.* § 1395y(a)(1)(A).

[3] CMS, Draft Guidance for the Public, Industry and CMS Staff: Factors CMS Considers in Opening a National Coverage Determination, at 4 (Mar. 9, 2005), *available at* http://www.cms.hhs.gov/mcd/ncpc_view_document.asp?id=1 [hereinafter NCD Guidance].

[4] CMS, Draft Guidance for the Public, Industry, and CMS Staff: Factors CMS Considers in Making a Determination of Coverage With Evidence Development (Apr. 7, 2005), *available at* http://www.cms.hhs.gov/mcd/ncpc_view_document.asp?id=2 [hereinafter Draft Guidance for CED].

[5] For examples of these coverage determinations, see CMS, Medicare Coverage Database, http://www.cms.hhs.gov/mcd/search.asp? (last visited Jan. 30, 2006).

[6] NCD Guidance, *supra* note 3.

[7] CMS, Fact Sheet: CMS Responds to Stakeholder Feedback Regarding Coverage with Evidence Development, at 1 (July 12, 2005), *available at* http://www.cms.hhs.gov/medicarecoverageguidedocs/downloads/guidfactsheet.pdf.

[8] Draft Guidance for CED, *supra* note 4.

[9] *Id.* at 4.

[10] 42 U.S.C. § 1395ff(f)(1)(B).

[11] Draft Guidance for CED, *supra* note 4, at 6.

[12] 42 U.S.C. § 1395y(a)(1)(A).

[13] Draft Guidance for CED, *supra* note 4, at 6.

[14] *Id.* at 7.

[15] CMS, Decision Memo for Anticancer Chemotherapy for Colorectal Cancer (CAG-00179N) (Jan. 28, 2005), *available at* http://www.cms.hhs.gov/mcd/viewdecisionmemo.asp?id=90; CMS, National Coverage Determinations Manual, ch. 1, § 110.17, *available at* http://63.241.27.79/manuals/103_cov_determ/ncd103c1_Part2.pdf (last visited Jan. 17, 2006).

[16] Draft Guidance for CED, *supra* note 4, at 8.

[17] FDG PET is a minimally-invasive diagnostic imaging procedure used to evaluate metabolism in normal tissue, as well as in diseases such as cancer. CMS, Decision Memo for Positron Emission Tomography (FDG) for Brain, Cervical, Ovarian, Pancreatic, Small Cell Lung, and Testicular Cancers (CAG-00181N) (Jan. 28, 2005), *available at* http://www.cms.hhs.gov/mcd/viewdecisionmemo.asp?id=92 [hereinafter FDG PET Decision Memorandum]; CMS National Coverage Determinations Manual, *supra* note 15, § 220.6.

[18] FDG PET Decision Memorandum, *supra* note 17.

[19] CMS, Decision Memo for Carotid Artery Stenting in Post-Approval Studies (CAG-00259N) (Oct. 12, 2004), *available at* http://www.cms.hhs.gov/mcd/viewdecisionmemo.asp?id=136.

[20] Draft Guidance for CED, *supra* note 4, at 9-10.

[21] *Id.* at 12.

[22] *Id.*

[23] *Id.* at 12-13.

[24] CMS Fact Sheet, *supra* note 7, at 2.

[25] *Id.* at 1-2; 42 U.S.C. § 1395ff(f)(2)(B).

[26] CMS Fact Sheet, *supra* note 7, at 4; CMS, Medicare Benefit Policy Manual, chs. 1, 14, 15, *available at* http://www.cms.hhs.gov/Manuals/IOM/itemdetail.asp?filterType=none&filterByDID=-99&sortByDID=1&sortOrder=ascending&itemID=CMS012673 (last visited Jan. 17, 2006).