Ethical Considerations in CMS's Coverage With Evidence Development

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This paper reviews the CMS coverage with evidence development policy, its manner of implementation, and key ethical issues raised by the policy. The author describes ethical considerations and issues associated with the process of coverage with evidence development for generating evidence for novel or emerging technologies.

Key Words: Coverage with evidence development, CMS, Medicare coverage, ethics

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New biomedical technologies—diagnostics, drugs, and devices—require scientific and regulatory assessment of benefits, risks, efficacy, and costs before being approved for patient care. Even after successfully navigating the extensive FDA approval process [1] and receiving coverage approval by local Medicare contractors, novel technologies may lack sufficient evidentiary support to justify issuance of a national coverage determination (NCD) by CMS. During the NCD process, scientific and medical evidence is gathered and weighed before a coverage decision (ie, payment or nonpayment) is made for the technology at issue. CMS will not authorize payment for service or devices "not 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 regulatory definition of "reasonable and necessary," but in 2000, CMS clarified that the primary factors in making an NCD include whether a technology is safe, effective, and appropriate and whether its use leads to improved meaningful health outcomes in the Medicare population [3]. Currently, cost-effectiveness is not a consideration in the coverage decisions.

Medicare has struggled with how best to provide timely and fair coverage for new but unproven medical technologies. On an ad hoc basis, some promising technologies that lacked sufficient outcomes data have received CMS coverage predicated on patient participation in prospective clinical trials or registries (Table 1 [4-9]). For example, the National Emphysema Treatment Trial, a multicenter, randomized trial begun in 1996, evaluated the relative value of lung-volume reduction surgery as an adjunct to medical therapy in patients with advanced emphysema [10]. The study is considered a successful collaborative model among federal agencies: the National Institutes of Health and the National Heart, Lung, and Blood Institute conducted the trial and CMS conditioned coverage on participation in their randomized controlled trial (RCT). Most important, the evidence from the National Emphysema Treatment Trial demonstrated that lung-volume reduction surgery should not be used for high-risk patients in inappropriate settings [11]. Since publication of the RCT's results in 2003 and the issuance of an NCD limiting payment to patients meeting criteria derived from the RCT, the number of lung-volume reduction surgery procedures being performed has dropped (Table 1).

IMPLEMENTING A POLICY OF COVERAGE WITH EVIDENCE DEVELOPMENT

In 2005, CMS issued draft guidance describing the policy of coverage with evidence development (CED) as an option whereby Medicare would cover new technologies and interventions even when there is insufficient evidence for CMS to make an NCD. Coverage with evidence development is specifically conditioned on eligible individuals' participation in relevant research or registries; coverage, or payment, is available only to those eligible individuals who agree to participate in the research [12]. The same year, CMS conditioned coverage for several medical technologies and interventions: off-label use of costly drugs for colorectal cancer in RCTs sponsored by the National Cancer Institute, enrollment in a registry for prospective data collection for prophylactic use of implantable cardioverter-defibrillators (ICDs), and enrollment in a prospective data collection registry for PET for cancers [13] (Table 1).

Responding to criticism and debate of the 2005 draft guidance, CMS issued a revised document, "National Coverage Determinations With Data Collection as a Condition of Coverage: Coverage With Evidence Development" [14]. The 2006 guidance document formalized and described two forms of CED, deriving statutory authority from the Social Security Act [2]: (1) coverage with

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Table 1. Coverage with evidence development

Technology Under Review*	Description	Outcome
LVRS (NCD 2003)	1996-2003 LVRS covered during NIH RCT (NETT)	2003 publication of RCT; LVRS completed
CREST (NCD 2001)	Category B investigational device exemption; coverage for enrollees in NINDS and NIH RCT	RCT ongoing
PET for dementia and neurodegenerative diseases (NCD 2004)	FDG-PET covered for enrollees with suspected dementia and enrolled in RCT or registry	PET registry trials ongoing
Chemotherapy for colorectal cancer (NCD 2005)	Off-label use of chemotherapy drugs for enrollees in NCI-sponsored RCTs	RCTs ongoing
Cochlear implantation (NCD 2005)	Coverage for enrollees in RCTs	No RCTs proposed
ICDs (NCD 2005)	Coverage for subgroup; prospective data in ACC National Cardiovascular Data Registry	Registry ongoing
PET for cancer (NCD 2005)	Coverage for providers, patients in registry; National Oncologic PET Registry	Registry ongoing
Home use of oxygen (LOTT) (NCD 2006)	RCTs; LOTT	RCTs ongoing
Artificial heart (NCD 2008)	RCT; category B investigational device exemption	RCT ongoing

Sources: ClinicalTrials.gov [4], Brott et al [5], CMS [6,8,9,13], National Oncologic PET Registry [7].
Note: ACC = American College of Cardiology; CREST = Carotid Revascularization Endarterectomy Versus Stenting Trial; FDG = 2-[^{18}F]fluoro-2-deoxyglucose; ICD = implantable cardioverter-defibrillator; LOTT = Long-Term Oxygen Treatment Trial; LVRS = lung volume reduction surgery; NCD = national coverage determination; NETT = National Emphysema Treatment Trial; NIH = National Institutes of Health; NINDS = National Institute of Neurological Disorders and Stroke; RCT = randomized controlled trial.
*Current as of March 2011.

appropriateness determination and (2) coverage with study participation (CSP). The intent of coverage with appropriateness determination is to provide coverage of a technology or service that meets the criteria of "reasonable and necessary" yet requires additional data to determine appropriateness criteria for the specific intervention [14]. Providers submit data to registries or databases specifically designed to aggregate the information needed to make an NCD, but the registries or databases are not necessarily monitored or maintained by CMS. The guidance document states that the data collection will be scientifically valid and that there will be patient safety monitoring, quality assurance, and data protection. Specifically addressing patient privacy concerns, CED databases, registries, and research protocols must be in compliance with provisions of 5 USC § 552(a) pertaining to records maintained on individuals [15], HIPAA [16,17], and protections of human research subjects [18].

Coverage with subject participation is analogous to the original CED concept. Where insufficient evidence exists to demonstrate that an item or service is adequate to meet 42 USC § 1862(a)(1)(A) standards, it could be considered "reasonable and necessary" if the patient enrolls in a clinical study designed to provide evidence of the risks and benefits of that of the item or service. If new evidence benefits diagnostic and therapeutic practice, and results are published in a peer-reviewed journal, the NCD would be reconsidered. Only patients who agree to be part of the study will be covered by CMS; nonparticipation will result in noncoverage. Statutory authority is cited at 42 USC § 1862(a)(1)(E), which permits Medicare coverage for items or services used in research determined to meet the priorities of Medicare by the Agency for Healthcare Research and Quality [19]. This second arm of CED has met with criticism as raising significant ethical questions.

ETHICAL ISSUES IN COVERAGE WITH EVIDENCE DEVELOPMENT

The federal policy for the protection of human subjects (the "common rule") incorporates principles of the Belmont report, and federally funded research is governed by those principles. The Belmont report, drafted in 1979, incorporates basic principles to guide the ethical conduct of research using human subjects [20]. The 3 prescriptive tenets of the Belmont report are justice or fairness, beneficence, and respect for persons or autonomy. The primary ethical questions raised by CED that touch on these tenets are (1) whether Medicare beneficiaries who either cannot, or will not, participate in a clinical trial are deprived of a right to coverage; (2) whether Medicare beneficiaries are an appropriate population to study and upon which to base coverage decisions; and (3) whether conditioned participation in a CED study is coercive.

Is CED equitable? Medicare beneficiaries are entitled to items or services that are "reasonable and necessary" and for which NCDs have been issued. Not all eligible Medicare beneficiaries have access to or consent to participation in CED research as a condition of coverage. Those beneficiaries lacking access to conditioned cover-