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Client Alert

FDA & Life Sciences and Healthcare Practice Groups

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CMS Issues New Draft Guidance on Coverage with Evidence Development Policy for National Coverage Determinations

On November 29, 2012, the Centers for Medicare & Medicaid Services (CMS) issued a new draft guidance regarding its Coverage with Evidence Development (CED) policy for National Coverage Determinations (NCDs). As CMS has increasingly used CED in recent years to provide, and often limit, Medicare coverage for promising new medical treatments, revisions to this policy will be of particular interest to those manufacturing or otherwise developing new and innovative technologies for which they seek Medicare coverage. Comments on the draft guidance may be submitted to CMS until January 28, 2013.

Background on CED

CED is an evidence-based coverage paradigm that permits CMS to develop coverage policies for certain items and services that are likely to show health benefits to Medicare beneficiaries but for which the available evidence base is not yet sufficiently developed.

CMS first published guidance on CED on July 12, 2006 in a document entitled, "National Coverage Determinations with Data Collection as a Condition of Coverage: Coverage with Evidence Development." That guidance set forth the parameters under which CMS would apply CED when issuing NCDs.

In the 2006 guidance, CMS described two different categories of coverage using CED: (1) Coverage with Appropriateness Determination and (2) Coverage with Study Participation. Coverage with Appropriateness Determination, based on Section 1862(a)(1)(A) of the Social Security Act, is used when the medical evidence is adequate to conclude that an item or service is reasonable and necessary for certain beneficiaries in certain circumstances, but additional data is required to demonstrate that the item or service is furnished as specified in the NCD. When applying Coverage with Appropriateness Determination, CMS required the establishment of data registries to which providers must submit clinical data regarding the items or services furnished to Medicare beneficiaries. The NCD for Implantable Cardioverter Defibrillators (ICDs), which linked expanded coverage of ICDs to a requirement to submit data to a national data registry, is an example of Coverage with Appropriateness Determination.

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Coverage with Study Participation, based on Section 1862(a)(1)(E) of the Social Security Act, is used when the medical evidence is not adequate to conclude that an item or service is reasonable and necessary, but coverage would be provided if the beneficiary were enrolled in a clinical study designed to provide additional medical evidence regarding the health risks and benefits of using the item or service. Pursuant to Section 1862(a)(1)(E), the clinical study is to be conducted pursuant to the authority of the Agency for Healthcare Research and Quality (AHRQ) to conduct and support research on outcomes, effectiveness, and appropriateness of services and procedures to identify the most effective means to prevent, diagnose, treat, and manage diseases, disorders and other health conditions. The NCD for Transcatheter Aortic Valve Replacement (TAVR), which permits coverage of the procedure for certain non-FDA approved indications in the context of CMS-approved clinical trials, is an example of Coverage with Study Participation.

On November 7, 2011, CMS solicited public comment on CED in an effort to make revisions to the 2006 guidance. CMS received approximately 50 comments, which requested that CMS address the following issues: (1) clear evidentiary criteria for applying CED, including when CED would end; (2) whether CED should be required only within the context of the coverage process; (3) whether local contractors should have the discretion to apply CED in local coverage; and (4) how CED could be applied in the context of FDA-CMS alignment.

In May 2012, CMS held a public meeting of the Medicare Evidence Development & Coverage Advisory Committee (MEDCAC) to consider the development of a more formalized evidentiary criteria for CED. The panel concluded the following:

- An evidentiary threshold can be defined by CMS to invoke CED;
- An evidentiary threshold can be defined by CMS to trigger an evidentiary review to determine if CED should cease, continue or be modified;
- An evidentiary threshold would be influenced by the general and particular characteristics of the item or service, the disease, and the availability of acceptable alternatives, and there may meaningful interaction of these characteristics;
- Generalizability (to additional settings, practitioners or other clinical indications) may comprise the primary evidence gap for some bodies of evidence; and
- Evidentiary standards do not remove the need for individuals to make situation specific judgments.

Application of CED to Future Coverage Decisions

Although CMS previously applied two different types of CED to coverage decisions, in the draft guidance, CMS states that the principal function of CED is to generate new evidence on the benefit or harm of an item or service among Medicare beneficiaries based on rigorous scientific activity. Therefore, because Coverage with Appropriateness Determination does not fundamentally encompass the concept of research, CMS will no longer use that term to describe CED. For future NCDs, CMS intends to consider only the form of CED allowing Coverage with Study Participation,

i.e., coverage when the evidence is insufficient to establish medical necessity but the beneficiary is enrolled in a clinical study conducted or supported by AHRQ. Thus, in the draft guidance, CMS defines CED as "a determination that an item or service is reasonable and necessary, based on the best available evidence, under an explicit condition that patients be enrolled in a research study that evaluates outcomes, effectiveness, and appropriateness of the item or service in question."

The draft guidance further proposes that CMS will review the following factors when applying CED to future coverage determinations for items and services:

- Relevance to health outcomes in the Medicare population (*i.e.*, where the available evidence is limited to narrow, but otherwise methodologically rigorous, protocols that did not adequately evaluate clinical outcomes relevant to affected Medicare beneficiaries);
- Representativeness of available evidence (*i.e.*, where the available evidence is not based on subjects who are representative of the affected Medicare beneficiary population);
- Evolution and reevaluation of evidence base (*i.e.*, where new evidence or reinterpretation of existing evidence calls into question past conclusions about the impact on patient health outcomes of a covered item or service); and
- Generalizability of study results to the Medicare population at large (*i.e.*, where evidence supporting the clinical benefit of a new item or service was developed in a setting that does not represent the typical community-based setting in which a Medicare beneficiary receives care).

CMS proposes that a MEDCAC meeting is not required for every instance in which the application of CED is considered, as such a requirement could effectively delay or prevent beneficiary access to items and services. Also, CMS recognizes that the alignment of CED with an FDA post-approval study requirement presents an opportunity for greater research efficiency.

Although the draft guidance does not specifically address the ability of Medicare contractors to apply CED in the context of Local Coverage Determinations, it recognizes the ability of contractors to cover items and services used in the context of clinical research studies pursuant to existing Medicare policies.

Clinical Studies under CED

The draft guidance proposes that CMS-approved CED studies must be designed and conducted prospectively to produce evidence to inform future Medicare coverage. CMS also expects that the results of all CMS-approved CED studies will be published in peer-reviewed journals. The following standards of scientific integrity and relevance to the Medicare population must be demonstrated in the clinical study:

- The principal purpose of the study is to test whether the item or service meaningfully improves health outcomes of patients who are represented by the enrolled subjects.

- The rationale for the study is well-supported by available scientific and medical information, or it is intended to clarify or establish the health outcomes of interventions already in common clinical use.
- The study results are not anticipated to unjustifiably duplicate existing knowledge.
- The study design is methodologically appropriate and the anticipated number of enrolled subjects is sufficient to answer the research questions being asked in the study.
- The study is sponsored by an organization or individual capable of completing it successfully.
- The study is in compliance with all applicable Federal regulations concerning the protection of human subjects found at 45 C.F.R. Part 46.
- All aspects of the study are conducted according to appropriate standards of scientific integrity set by the International Committee of Medical Journal Editors.
- The study has a written protocol that clearly demonstrates adherence to Medicare requirements for CED.
- The study is not designed to exclusively test toxicity or disease pathophysiology in healthy individuals, except if the disease or condition being studied is life threatening as defined in 21 C.F.R. § 312.81(a) and the patient has no viable treatment options.
- The study is registered on the ClinicalTrials.gov Website and/or the Registry of Patient Registries by the principal sponsor/investigator prior to the enrollment of the first study subject.
- The study protocol specifies the method and timing of public release of results on all pre-specified outcomes, including release of negative outcomes, which must be made public within 24 months of the end of data collection. If a report is planned to be published in a peer reviewed journal, then initial release may be an abstract that meets the requirements of the International Committee of Medical Journal Editors, and a full report of the outcomes must be made public no later than three years after the end of data collection.
- The study protocol explicitly discusses subpopulations affected by the item or service under investigation, particularly traditionally underrepresented groups in clinical studies, how the inclusion and exclusion criteria affect enrollment of these populations, and a plan for the retention and reporting of said populations in the trial.
- The study protocol explicitly discusses how the results are, or are not, expected to be applied to subsections of the Medicare population to infer whether Medicare patients may benefit from the intervention.

Ending CED

The draft guidance proposes that the application of CED for identified uses of an item or service will end when one of the following conditions are met: (1) no CED studies are approved within the timeframe required by the CED decision;

(2) approved CED studies are not completed within the timeframe required by the CED decision; or (3) CED studies are completed. CMS recognizes that there may be a period of noncoverage between the end of the CED study and CMS's review of the study results, although CMS intends to work with investigators to develop integrated research strategies that allow continuation of coverage when a randomized clinical trial ends, where appropriate.

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The draft guidance may be found by clicking here.