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What's New in GCP?
CMS Revises IDE Coverage Rules

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The Centers for Medicare and Medicare Services (CMS) revised its regulations to allow coverage for routine care items and services for investigational device exemption (IDE) clinical studies but delayed implementation of the changes until Jan. 1, 2015.

Coverage will be allowed for both Category A and B IDE studies if CMS or its designated entity (for Category B) determines the study has an FDA approval letter of the IDE, an IDE study protocol, an institutional review board approval letter, and a National Clinical Trial (NCT) number and supporting materials, if needed. The studies also must meet 10 criteria:

- The principal purpose of the study is to test whether the device improves health outcomes of appropriately selected patients.
- 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 adequate to confidently answer the research question(s) being asked in the study.
- The study is sponsored by an organization or individual capable of successfully completing the study.
- The study is in compliance with all applicable federal regulations concerning the protection of human subjects found at 21 C.F.R. Parts 50, 56 and 812, and 45 C.F.R. Part 46.
- Where appropriate, the study is not designed to exclusively test toxicity or disease pathophysiology in healthy individuals. Studies of all medical technologies measuring therapeutic outcomes as one of the objectives may be exempt from this criterion only if the disease or condition being studied is life threatening and the patient has no other viable treatment options.
- The study is registered with the National Institutes of Health's National Library of Medicine's ClinicalTrials.gov.
- The study protocol describes the method and timing of release of results on all pre-specified outcomes, including release of negative outcomes and that the release should be hastened if the study is terminated early.
- The study protocol must describe how Medicare beneficiaries may be affected by the device under investigation and how the study results are or are not expected to be generalizable to the Medicare beneficiary population. Generalizability to populations eligible for Medicare due to age, disability or other eligibility status must be explicitly described.

More Criteria Needed for "Appropriate" Study Design for Medicare Beneficiaries

"We believe that additional Medicare coverage criteria are needed for Category A and B IDE studies where Medicare coverage for items and services is sought, to ensure that the study design is appropriate to answer questions of importance to the Medicare program and its

beneficiaries," the agency said in announcing the rule change. "The use of such a device in an IDE study or trial may expose study participants to increased risks that must be balanced by other factors, including the likelihood that the study would add important information to the body of medical knowledge relevant to the Medicare program."

The agency noted that "the protocol is the primary source of knowledge on the proposed design and management of the study. Without this document, reviewers and funding entities are unable to ascertain the quality and validity of the study, and whether the study is appropriate to answer questions of importance to the Medicare program."

The agency added that they "expect the results of all approved studies will specifically benefit the Medicare population and, as such, covered studies or trials must address how the study will affect Medicare beneficiaries if it desires to receive Medicare payment for services provided to Medicare beneficiaries within the study."

CMS said "numerous studies that may be considered scientifically valid are of little benefit to the Medicare program. We are sensitive to the unique needs of Medicare beneficiaries, particularly the elderly. A trial design that may be adequate for a generally younger population may be comparatively insensitive to clinical factors commonly found in the elderly that may adversely impact the potential benefit of tolerability of a device, which is of particular importance to the Medicare program."

As part of the revision, CMS now divides IDEs into Category A (Experimental) and Category B (Nonexperimental/investigational) and clarified the Category B IDE definition as "a device for which the incremental risk is the primary risk in question (that is, initial questions of safety and effectiveness of that device type have been resolved, or it is known that the device type can be safe and effective because, for example, other manufacturers have obtained FDA premarket approval or clearance for that device type.)"

In addition, the definition of routine-care items and services states that those "are otherwise generally available to Medicare beneficiaries (that is, a beneficiary category exists, it is not statutorily excluded, and there is no national noncoverage decision) that are furnished during a clinical study and that otherwise would be furnished even if the beneficiary were not enrolled in a clinical study."

The revised regulation also states that a listing of all CMS-approved Category A and B IDE studies will be posted on the CMS website and published in the Federal Register. "We encourage providers to check the CMS website to see if an IDE study has been approved for coverage before submitting IDE-related claims," CMS said.

CMS Settles on Central Review

Once the regulation revision is in effect, sponsors seeking Medicare coverage for IDE studies must submit requests via email or hard copy to CMS, which will be the centralized point of contact for submission, review and determination of Medicare coverage IDE study requests.

"Providers will no longer need to notify individual contractors regarding IDE studies for which they plan to submit claims," the agency noted. CMS added that "a centralized review process would be more efficient by reducing the burden for stakeholders interested in seeking Medicare coverage related to nationwide IDE studies or trials. Having a single entity making Medicare coverage decisions would enhance administrative efficiency by eliminating the need for duplicative submissions from stakeholders to different Medicare contractors and duplicative reviews by Medicare contractors."

Comments on the proposed rule contended that the old process of review by local Medicare contractors was still appropriate for small, single-site studies and centralized review should only be applied to large, national studies.

They asserted that the coverage requirements "would increase burden and create access barriers for Medicare coverage…decelerate medical device innovation and that many sponsors may choose not to seek Medicare coverage for IDE trials."

CMS noted that seeking Medicare coverage is voluntary and that "study sponsors are not required to seek Medicare coverage in order to conduct their studies or trials. Establishing separate Medicare coverage for IDE study review processes for large and small studies would create unnecessary infrastructure," the agency said.

The requests for coverage must include a request letter that describes the scope and nature of the IDE study, discussion of how the IDE study meets each Medicare Coverage IDE Study Criteria, the FDA approval letter of the IDE, the IDE study protocol, the IRB approval letter, the NCT number, and supporting materials, as appropriate.

CMS said it expected to be able to review complete requests in about 30 days. "While we believe that we have sufficient resources to process Medicare coverage reviews of the IDE studies," the agency said that it is modifying the regulations "to allow for reviews by a CMS-designated entity if future needs arise."

Noting that some comments to the proposed regulations contended the Medicare coverage requirements duplicated the FDA's requirements, the agency said the FDA "approves IDE studies or trials when, among other things, the risks to the subjects are outweighed by the anticipated benefits and the importance of the knowledge to be gained. For purposes of Medicare coverage, we seek evidence that an item or service is reasonable and necessary. The disease burden borne by elderly individuals and the important health care interventions unique to the Medicare population are important areas of focus for the Medicare program; we would not expect the FDA review to include substantive consideration of these Medicare priorities. Thus, we believe that Medicare coverage standards are needed for IDE studies for which Medicare coverage is sought," CMS said.

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