



Statement

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AAMC Responses to MCAC Questions Worksheet

Presented by

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Centers for Medicare and Medicaid Services

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My name is Bryan Soronson. I am Senior Administrator for the Department of Neurology, University of Maryland School of Medicine. I have been managing the administrative and financial aspects of clinical trials for over 25 years. My testimony today is presented on behalf of the Association of American Medical Colleges, representing all 125 accredited U.S. and 17 accredited Canadian medical schools; nearly 400 major teaching hospitals and health systems, including 68 Department of Veterans Affairs medical centers; and 96 academic and scientific societies.

Just over 6 years ago CMS began covering the routine costs of Medicare beneficiaries enrolled in clinical trials. This new policy was thought to have much promise for both beneficiaries and the research community. However, for providers—both hospitals and physicians---the policy has been rife with confusion and its implementation has been thwarted by fears that the inadvertent submission of an incorrect bill could have dire results. I am pleased that CMS recognizes that the policy needs to be re-examined and is soliciting comments from those who are directly affected.

The goals of a revised Medicare clinical trials policy should be clarity, ease of implementation, and consistency among Federal agencies that are involved in clinical research, primarily the Food and Drug Administration and the National Institutes of Health. Additionally, other parts of CMS must coordinate their efforts to address issues related to clinical trials that are outside the scope of the MCAC's authority. For instance, due to cost sharing requirements, the current system discourages Medicare Advantage beneficiaries from enrolling in clinical trials. Also of much concern is the interplay between the clinical trials policy and Medicare Secondary Payer rules. Until these issues are addressed, the clinical trials policy will not attain the necessary clarity and ease of implementation that are needed to ensure its success.

Given the time constraints, I will use the remainder of my time to respond to the questions posed in the MCAC Questions Worksheet.

1. How should CMS define a good clinical study?

The AAMC strongly supports Option 1.c., endorsing external sources. The FDA *Guidance on General Considerations for Clinical Trials* is the most authoritative source. Its contents are well-known to the research community, and its adoption by CMS as the standard for judging a good clinical study will be an important step toward achieving consistency among Federal agencies. To ensure continued consistency between CMS and the FDA, it is important that CMS provide assurance that if FDA makes changes to this policy, they will be adopted by CMS.

2.a. Should these two current standards (i.e., therapeutic intent and enrolling patients with diagnosed disease rather than healthy volunteers) remain?

As currently stated, the two criteria are confusing. The first bullet makes the definitive statement that a clinical trial “must have therapeutic intent,” while the second bullet implies that trials of diagnostic interventions also may be covered.

The AAMC suggests that in addition to paying for the routine costs of trials with “therapeutic intent,” Medicare pay the routine costs of beneficiaries participating in trials of diagnostic interventions. These trials are important because they can lead to earlier detection of conditions, when treatments are most likely to be beneficial. It is even possible that such trials would be covered by Medicare under the Coverage with Evidence Development (CED). Therefore, we suggest that the standard be revised to read:

- The study must not be designed exclusively to test toxicity or disease pathophysiology. It must either have therapeutic intent or be a trial of a diagnostic intervention under a CED.
- Trials with therapeutic intent must enroll patients with the diagnosed disease. Trials of diagnostic interventions may enroll healthy patients in order to have a proper control group. Medicare will pay the routine clinical costs for all individual enrolled in these trials, including healthy patients.

2.b. Definition of therapeutic intent

The proposed CMS definition of “therapeutic intent” forecloses the possibility of coverage for any Phase I studies. The AAMC supports Medicare coverage of certain Phase I studies, particularly those of cancer treatments, analogous to our support of Medicare coverage of humanitarian use devices that have HDE status. Therefore, we suggest that the definition of therapeutic intent be revised as follows:

A qualified study exhibits therapeutic intent when a major objective of the study is the diagnosis or treatment of disease, including the observation of benefit of the intervention under study. A Phase I study that enrolls Medicare eligible patients with the disease under study and has as an objective the diagnosis or treatment of the disease shall be considered to have therapeutic intent.

You also asked whether CMS should “define therapeutic intent differently for studies evaluating diagnostic services.” The evaluation of a diagnostic service does not have therapeutic intent, though as was stated above, Medicare should cover the routine costs of patients enrolled in these studies. CMS should make clear that these studies will be covered, provided that they meet the criteria set forth in Question 2.b.

Question 2.b., 5 Medicare-specific standards. The AAMC has the following comments on each of these standards:

1. The AAMC strongly supports requiring the registration of trials on the ClinicalTrials.gov website.
2. While the AAMC strongly supports requiring the public release of study results, there currently exists no publicly supported and operated site where such information can be reported. Some drug manufacturers report results of some or all of their clinical trials on their company websites, but the results are not subject to any form of external review. Should the appropriate Federal agencies work together to create a site for posting trial results that is similar to ClinicalTrials.gov, then it would be appropriate for Medicare to consider requiring results reporting as a condition of Medicare coverage for beneficiaries participating in these trials. To impose such a requirement now would, in our judgment, be premature.

3. The AAMC supports requiring an explicit discussion of the consideration of relevant subpopulations in the study protocol.
4. It is extremely important that prior to enrolling Medicare beneficiaries in a clinical trials, both providers and the beneficiaries themselves know whether Medicare will cover the routine clinical costs of the trial. However, depending on trial purpose and design, it may not be possible until a trial is completed to determine if sufficient Medicare populations have been included. As proposed, standard 4 seems to open the possibility that Medicare could retroactively deny coverage for a clinical trial because it did not recruit a sufficient number of Medicare beneficiaries. Such a policy would serve as a barrier to enrolling Medicare beneficiaries in clinical trials.

Of greater concern is that the proposed Standard 4. attempts to limit Medicare coverage to those studies that are designed specifically to enroll a statistically valid Medicare population. Many significant pathologies that afflict Medicare beneficiaries, such as high blood pressure and type II diabetes, have their onset long before individuals become eligible for Medicare, and require treatments that extend throughout an individual's Medicare-eligible years. Typically, studies of these conditions seek to recruit a broad spectrum of the population that may certainly include, but not be especially directed at, Medicare-eligible participants. The knowledge gained from these studies may be of enormous benefit to Medicare enrollees, as well as to younger populations. It is a very short-sighted view that does not serve the Medicare population well to exclude such studies from Medicare coverage. Moreover, to adopt the proposed standard could well have the perverse consequence of deterring enrollment of Medicare beneficiaries in studies that may be of great benefit to them.

We suggest that that a more appropriate criterion would be as follows:

The study is of a disease that is known to affect the Medicare population and it includes a discussion of reasonable efforts that will be made to enroll Medicare beneficiaries.

5. The AAMC supports the use of any standard required through a national coverage determination using CED.

The AAMC requests that CMS clarify whether a study must meet all 5 standards to qualify for Medicare coverage.

Question 3. Deeming for “good clinical studies”

The AAMC supports the 4 criteria listed. We ask that CMS clarify that meeting any one of the 4 criteria will qualify a study for “deeming.”

Question 4. IND Exempt studies

The AAMC supports allowing IND Exempt studies to be deemed if they meet any one of the 4 criteria set out in Question 2.b.

Question 5. Deeming of studies approved ?but? not funded by a Federal agency.

The AAMC supports the deeming of these studies only if they meet any one of the 4 criteria set out in Question 3.

Question 6. Additional methods to approve studies for Medicare coverage.

1. Any study required through a national coverage determination using CED is most desirable.
2. The other 3 suggestions (a Federal inter-agency panel to review protocols, establishing a multi-stakeholder panel to review protocols, or working with other Federal agencies to incorporate their scoring process) are least desirable. The lack of details about how any of these processes would work is of concern, as is the difficulty of being able to assemble a sufficient number of people with the requisite expertise in a reasonable amount of time. There would be little or no value to adopting a process that slows clinical research by imposing new bureaucratic requirements.

Question 7. Clarification of the definition of “routine clinical services.”

The first criterion of “routine clinical services” is that such items and services are “available to Medicare beneficiaries outside of a clinical study.” Within the medical community, such items and services are commonly referred to as “standard of care” or “conventional care.” We recommend that CMS adopt these terms since they already are widely used and understood, and thus will provide greater clarity for those implementing this policy. CMS may have concerns that “standard of care” or “conventional care” could vary geographically, but that would be equally true if these items and services are characterized as being available outside of a clinical study. For further clarity, we also recommend that the latter part of the sentence be rewritten as follows:

. . . , not including items or services that meet the definition of investigational clinical **or administrative** services. (bold indicates added language)

The second criterion is that the items and services are “used for patient medical management within the study.” The meaning of “patient medical management” is unclear. If the purpose of this term is to reference 42 CFR §410.32(a), *Ordering Diagnostic Tests*, then it would add clarity to revise the criterion as follows:

b. Diagnostic tests that comply with the requirements of 42 CFR §410.32(a).

The three remaining criteria are reasonable and should be adopted as part of the definition of “routine clinical services.”

Question 8a. Definition of Administrative services.

The AAMC supports this definition and notes that the items comprising “administrative services” typically are paid from the study budget.

Question 8b. Definition of investigational clinical services.

The AAMC supports the proposed definition of “investigational clinical services” and the three exceptions that would allow coverage.

Again, thank you for this opportunity to comment on your proposed revisions to the clinical trials policy. I would be pleased to answer questions.

