Guidance for the Public, Industry, and CMS Staff
Coverage with Evidence Development
Document Issued on November 20, 2014

Appendix: Summary of Public Comments (received 11/29/12-1/28/13) and Responses

CMS received 27 public comments on the draft guidance posted on November 29, 2012. This appendix to the final guidance summarizes and responds to the major themes of the public's comments. In general, commenters were supportive of Coverage with Evidence Development; however, there were particular aspects of the draft guidance with which commenters disagreed.

Comment: Numerous commenters suggested that the eight principles governing the application of CED be reinstated in the revised guidance document. Commenters stated that by failing to restate the principles, it was unclear which principles the agency believed were no longer applicable and which ones would still apply.

Response: We appreciate the comment and we have included seven of the original eight principles in the final guidance document. One principle has been modified to state more generally that CED is accomplished through the coverage determination process, which may occur within the NCD or LCD processes. We have deleted the principle stating that CMS expects to use CED infrequently. The removal of this principle is not intended to signal that the agency expects to issue a flood of CED decisions. Rather, since 2006, we have found that the nature of NCD requests has changed. Technology is being presented to the agency for coverage earlier in the technology lifecycle, and requesters more frequently ask CMS to consider using CED for their particular technology. With these changes, it is no longer appropriate to include as a guiding principle our expectation that CED will be used only "infrequently."

Comment: Commenters requested that we refrain from duplicating the efforts of other agencies or asking for evidence that is duplicative of existing evidence; and that we use CED to expand coverage of technologies.

Response: We agree, and by restating in the final guidance document the principles governing the application of CED, we are agreeing with many commenters who expressed concerns that CED should not be used when other forms of coverage are justified, should not duplicate efforts of other agencies, should not be used to duplicate existing medical evidence, and should generally expand access to technologies.

Comment: We received many comments regarding the role that Local Coverage Determinations (LCDs) would have in CED. One commenter supported CED decisions through LCDs, while the remaining commenters only supported CED through National Coverage Determinations (NCDs).

Response: Although the definition of local coverage determination (LCD) in the Social Security Act does not support the use of CED under 1862(a)(1)(E) of the Act, Medicare Administrative Contractors (MACs) may use LCDs to determine coverage of items and services to the extent that they do not conflict with national Medicare policy. We are confident that the LCD process is open and transparent, allowing public comment on draft policies and thereby providing opportunity for input from the stakeholders that would be impacted by a CED decision. The Agency will work toward providing CED guidance to the MACs.

Comment: A commenter suggested that coverage under 1862(a)(1)(A) and 1862(a)(1)(E) should be simultaneous; while another commenter stated that coverage should be available both "with research" and "in research," suggesting that a technology covered under an NCD with CED should also be covered when furnished outside of a research study.

Response: Both statutory authorities cited by the commenters allow for CED. However, we disagree with the commenters' suggestion that, under CED, an item or service may be covered for beneficiaries within a clinical study and, at the same time, be covered for beneficiaries outside the clinical study. When CED under section 1862(a)(1)(E) is required, it is because there are outstanding questions about the health benefit of the item or service in the Medicare population. As such, the item or service is covered only in the context of a study that requires patient monitoring, data collection, and an open presentation of results. When CED under section 1862(a)(1)(A) is required, it is because additional clinical information is needed to ensure the appropriate use of the item and service in the Medicare population to facilitate accurate claims processing and payment.

Comment: Some commenters asked that we identify standards to distinguish the evidentiary requirements between studies of items and services that are for diagnosis and treatment.

Response: Although trial designs may differ, studies of both diagnostic services and treatments should include research questions related to health outcomes.

Comment: Some expressed concern about the burden of CED and encouraged the agency to require the collection of the minimum necessary data to address the research questions in addition to considering many types of study designs including the use of observational databases.

Response: We agree that, in general, the least burdensome approach to answering the CED questions would be appropriate.

Comment: Commenters also discussed concerns about patient access to technologies. Some commenters stated that CED should not be applied to long-covered technologies, medically accepted indications or technologies under FDA post approval studies.

Response: We do not agree. There are many standing NCDs for items and services for which evidence has never been established. CMS may find it appropriate to reconsider an NCD and require CED to fully consider the effectiveness of some of these technologies.

Comment: Some public commenters are looking for CMS to further engage stakeholders when it comes to CED decisions with earlier notification regarding when CMS is considering CED and collaboration with additional stakeholders including private health insurance plans and Medicaid.

Response: The NCD process is open to public comment. We post a tracking sheet announcing to the public when we are reviewing an item or service. All stakeholders are encouraged to comment on the tracking sheet. These comments assist us in developing our proposed NCD. There is a second public comment period after we issue the proposed NCD and before we issue the final NCD. We again use these comments to refine our proposed NCD. We believe the current process provides ample opportunity for the public to provide their views and for CMS to consider them.

Comment: Overall, commenters sought more details regarding when CED will be invoked and what evidence will be required for CED to end.

Response: The guidance document includes a general discussion about when we believe CED should end. However, we note that individual CED decisions reflect the specific technology and the state of the evidence, neither of which can be generalized to all technologies. It would be impracticable to set an across-the-board threshold for when to invoke or end CED. Such thresholds inevitably would not be relevant to all technologies and would not allow the necessary flexibility to tailor the CED approach when needed. We anticipate ending CED when appropriate, as discussed in the guidance.

Comment: Some commenters would like the agency to agree that the ending of CED should be based on pre-specified endpoints and agreed upon by the study investigators. Some commenters expressed concern about coverage during the time period between when a CED study ends enrollment and when CMS completes a reconsideration of the CED coverage decision.

Response: In each of the NCDs requiring CED, CMS identifies the research questions pertinent to the particular CED policy. Studies with a specific design, such as randomized clinical trials, have established start and end dates. When enrollment and follow up are complete, the data are to be analyzed and published in the peer reviewed medical literature. We expect that the studies conducted under a CED NCD will produce evidence that will lead to revisions to Medicare coverage policies, such as to the NCD that included CED as a component of the decision (for example, NCDs for oncologic uses of FDG PET and ventricular assist devices).

When an NCD requires CED under 1862(a)(1)(E), it is because the available evidence about a particular item or service is insufficient to cover it in the Medicare population outside the context of a well-designed clinical research study. While CMS does not believe that beneficiaries should have broad access to these items or services when scientific results are unavailable, there are ways to avoid or minimize the gap between the end of clinical studies under a CED NCD and revised coverage based on the results of CED studies. As discussed in the guidance, sponsors should build interim analyses into their study design and communicate these results to CMS. If the results support consideration of a change in the coverage status of the item or service, a revised NCD could be expedited.

Comment: Commenters requested clarification of AHRQ's role in CED.

Response: For CED NCDs issued under section 1862(a)(1)(E), AHRQ reviews and approves the CED questions and general standards for CED studies.