

Coverage with Evidence Development Proposed Revised Requirements

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Impact of Coverage with Evidence Development (CED)

- Since 2005 CED has facilitated access to 27 therapies when there was uncertainty regarding long-term outcomes, how well the therapy worked in real-world practice settings, durability, and subpopulation effects.
- The 2014 guidance document defined the scope and helped to clarify the statutory basis, scope, requirements, and evidentiary expectations of CED studies.
- Current proposed revised requirements reflect an opportunity not just to modernize CED, but also to make the process more transparent, predictable, and timely.

Timeliness of Reconsiderations

- There are ongoing discussions of modernizing Medicare coverage processes for novel technologies and how advancements in real world evidence (RWE) generation strategies may substantially impact the use of CED.
- A growing number of novel technologies are approved with limited evidence on long term outcomes, durability, and other evidence that informs patient selection and treatment success in real-world practice.
 - Many of these products may reach FDA approval with limited evidence to substantiate “reasonable and necessary” determination for Medicare coverage.
 - Clinical trial populations may not be not representative of a real-world Medicare patient population. Continued evidence development post-approval can inform the value of novel technologies, which can be challenging without coverage.

Timeliness of Reconsiderations

- Concurrent with the growing pace of medical innovation are the growing importance of real world data (RWD) and real-world evidence (RWE) as a means to evaluate health outcomes for Medicare beneficiaries.
 - New RWE methods are becoming a more efficient means of generating the evidence that could substantiate the construct of “appropriate for use in Medicare beneficiaries” in Medicare’s definition of “reasonable and necessary.”
- The proposed revised requirements will support innovative RWE generation strategies that support fit-for-purpose studies, allowing CMS to reevaluate efficient and appropriate coverage of novel technologies for Medicare beneficiaries in a predictable, transparent, and timely manner.

Encouraging Innovative RWD Collection Strategies

- Real world data must be reliable, relevant, and high quality to be conclusive and help CMS determine if a product is performing as expected in real-world settings and in the intended populations.
- The proposed revised requirements on data generalizability, robustness, completeness, and accuracy are important additions to ensuring data relevancy and quality, and will help investigators design rigorous real-world studies while allowing CMS to more confidently interpret study results.
 - Further, the proposed revised data quality and transparency requirements will help investigators design studies that will collect and analyze high-quality data with a level of transparency that can allow for replication.
- Finally, the proposed revised requirements targeting data validity, relevancy, and accuracy will contribute to the degree of confidence that CMS can derive from CED study results.
 - Collecting data that is representative and generalizable to the population of interest is a key element of data relevancy and will support CMS's goals of ensuring generalizability to the Medicare population.

Defining Appropriate Use with RWD

- Understanding how a technology performs for patients in “usual sites of care” can help CMS determine the appropriateness of a technology for Medicare beneficiaries.
- The proposed revised requirements allow CMS to set provider, site, or patient criteria when patient safeguards are needed, but also allow for data collection to reflect:
 - Changes in sites of care and intended populations over time,
 - Wider availability and thus experience with the technology, and
 - Differential data collection capabilities and capacities across different sites of care.
- Ultimately, the proposed revised requirements may allow CMS to inform standards and guidelines for use of novel RWD sources for use in CED studies.

Early Engagement to Inform Study Objectives and Design

- In order to reduce patient, provider, and sponsor burden, post-market studies could be designed to meet both FDA and CMS data collection requirements, which can be achieved through early engagement across sponsors and both agencies.
- Investigators may need additional guidance from CMS on outcomes of interest, study duration, and data collection efforts to design an effective study that would generate the types of evidence CMS would need to end a CED.
- Proposed revised requirements will support early engagement between CMS, sponsors, FDA, and other stakeholders and will allow CMS to:
 - Efficiently identify evidence gaps relevant to Medicare beneficiaries,
 - Provide guidance on CED study designs and study milestones, and
 - Complete the CED process in a timely and predictable manner.

Increasing CMS Resources

- With the growing pace of novel technologies, the ratio of CED to total coverage decisions continuing to increase, and the number of interactions needed to reach consensus on the types of evidence that would support fit-for-purpose studies increasing, CMS will need more resources to engage with sponsors and provide guidance on important outcomes of interest.
- Any new CED requirements and new coverage pathways will have the greatest impact if accompanied by steps to ensure adequate resources and capacity for CMS implementation.
- The additional resources to support coverage activities will be modest relative to their impact on innovation and Medicare patients' health outcomes.

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