Medicare Evidence Development & Coverage Advisory Committee

Reconsideration of the CMS Clinical Trial Policy

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CTP Reconsideration

- Standards
- Process for ensuring standards are met
- Covered Services

Issues not discussed

- Medicare Advantage plans
- Medicare secondary payer rules & regulations
- Part D

Current Standards

Clinical Trial Standards

3
Requirements

7 Highly
Desirable
Characteristics

3 Requirements

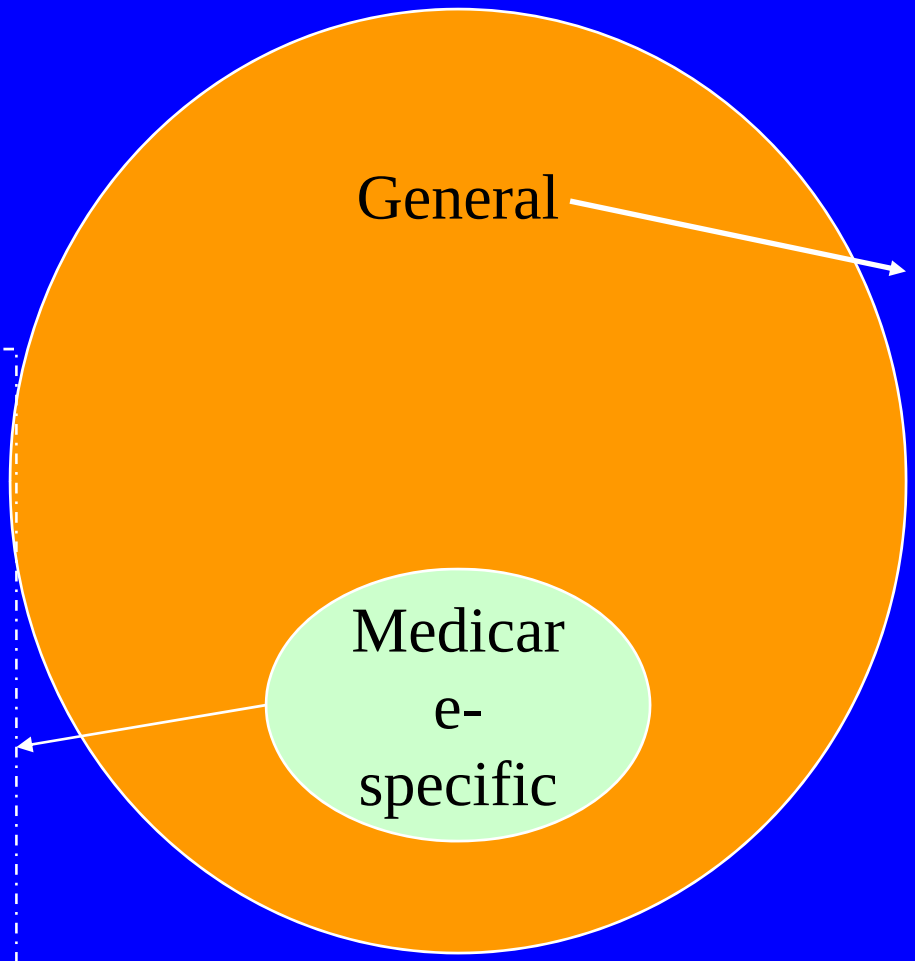
- The subject or purpose of the trial must be the evaluation of an item or service that falls within a Medicare benefit category (e.g., physicians' service, durable medical equipment, diagnostic test) and is not statutorily excluded from coverage (e.g., cosmetic surgery, hearing aids).
- The trial must not be designed exclusively to test toxicity or disease pathophysiology. It must have therapeutic intent.
- Trials of therapeutic interventions must enroll patients with diagnosed disease rather than healthy volunteers. Trials of diagnostic interventions may enroll healthy patients in order to have a proper control group.

7 Highly Desirable Characteristics

- The principal purpose of the trial is to test whether the intervention potentially improves the participants' health outcomes;
- The trial 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 trial does not unjustifiably duplicate existing studies;
- The trial design is appropriate to answer the research question being asked in the trial;
- The trial is sponsored by a credible organization;
- The trial is in compliance with Federal regulations relating to the protection of human subjects; and
- All aspects of the trial are conducted according to the appropriate standards of scientific integrity.

Proposed Standards

Clinical Trial Standards



- Narrative definition
- Current definition
- Reference definition

- Therapeutic Intent
- Patients with disease
- Trial Registration
- Publish Results
- Minority Disparities
- Age Representation
- CED Standards

General Standards

- Narrative definition
- List of characteristics
- Reference standard

Definition of Good Clinical Study (1)

A clinical trial is any investigation in human subjects intended to discover or verify the clinical effects of an investigational product or procedure, and to identify any adverse reactions to an investigational product or procedure with the object of ascertaining its safety and effectiveness. Procedures to assure that the rights, safety, and wellbeing of trial subjects are protected; consistent with the principles that have their origin in the Declaration of Helsinki must be followed.

Definition of Good Clinical Study (2)

Clinical research is the observation of events in groups of individuals who share a particular characteristic, such as a symptom, sign or illness; or a treatment or diagnostic test provided for the symptom sign or illness. Inferences are made based on comparisons of rates of predefined outcomes among groups. Procedures to assure that the rights, safety, and wellbeing of study participants are protected; consistent with the principles that have their origin in the Declaration of Helsinki must be followed.

List of Qualities of Good Clinical Study (Current Definition)

A good clinical study includes the following attributes:

- The principal purpose of the study is to test whether the intervention potentially improves the participants' health outcomes;
- 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 does not unjustifiably duplicate existing studies;
- the study design is appropriate to answer the research question being asked in the study;
- the study is sponsored by a credible organization or individual capable of executing the proposed study successfully;
- the study is in compliance with Federal regulations relating to the protection of human subjects; and
- all aspects of the study are conducted according to the appropriate standards of methodological and scientific integrity.

Reference Standards

- FDA
- Others

Medicare-specific Standards

1. The subject or purpose of the trial must be the evaluation of an item or service that falls within a Medicare benefit category (e.g., physicians' service, durable medical equipment, diagnostic test) and is not statutorily excluded from coverage (e.g., cosmetic surgery, hearing aids).

- This is a legal requirement and not a study standard and, while remaining part of the policy, will not be listed as a standard.

Medicare-specific standards

2. The trial must not be designed exclusively to test toxicity or disease pathophysiology. It must have therapeutic intent.

- Definition of therapeutic intent: A qualified trial exhibits therapeutic intent when a major objective of the study seeks as its goal the diagnosis or treatment of disease including observation of benefit of the intervention under study.

Medicare-specific standards

3. Trials of therapeutic interventions must enroll patients with diagnosed disease rather than healthy volunteers. Trials of diagnostic interventions may enroll healthy patients in order to have a proper control group.

- We propose to clarify the above criteria by stating that," trials of therapeutic intent may assign patients to a control group."

Proposed Medicare-specific Standards

1. All studies must be registered in ClinicalTrials.gov prior to patient enrollment (NCT# required on claim).
2. Protocol must specify method and timing of public release of results regardless of outcome or completion of trial.
3. Standards required in NCD using CED.

Proposed Medicare-specific Standards

4. The study must have explicitly discussed consideration of gender, race/ethnicity, age or other relevant subpopulations in the study protocol.
5. If the study results are to be used to inform Medicare coverage policy, the study must contain an explicit discussion of how the enrollment process will ensure that sufficient Medicare populations are included to clinically and statistically determine that Medicare populations benefit from the intervention.

Process

Current Process

Process for Ensuring Standards Are Met
- Current Policy -

3 Requirements

None specified.
Contractors involved sporadically.

7 Highly Desirable Characteristics

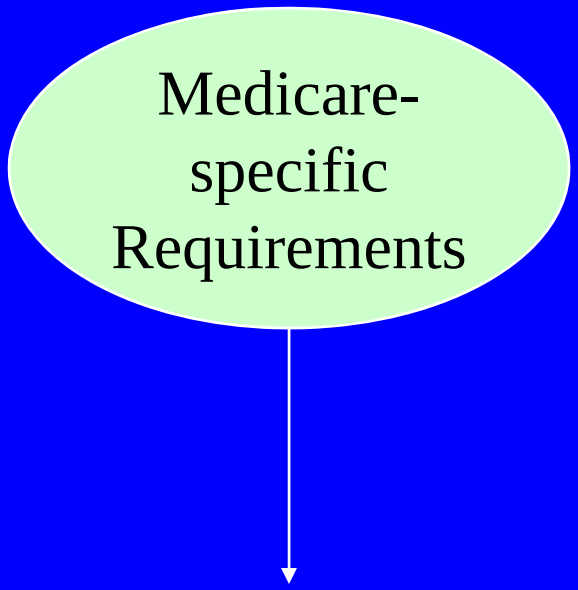
Deemed to meet standards if:

- Funded by specific Federal agency
- Supported by center/group funded by specific Federal agency
- IND
- IND Exempt (temporarily)

Self-certification (never implemented)

Proposed Process for Medicare-specific Standards

Process for Ensuring Medicare-specific Standards Are Met



Proposed Changes to Process for Ensuring General Standards are Met

Process for Ensuring General Standards Are Met

- Expand “deemed” status to all Federal agencies.
- Add to “deemed” status a requirement that the study be reviewed and approved as meeting that agency’s standards for a good study.
- Continue “deemed” status for:
  - Studies reviewed, approved, and funded by a Federal agency
  - Studies conducted by centers/groups supported by a Federal agency that has reviewed and approved the study
  - IND studies
- Remove “deemed” status from IND Exempt studies.
- Remove self-certification

Process for Ensuring General Standards Are Met - Potential Options

- Add “deemed” status for FDA approved post-approval studies.
- Study required through a NCD using CED.
- Establish a Federal inter-agency panel to review study protocols.
- Establish a multi-stakeholder panel to review study protocols.
- Work with other Federal agencies to incorporate into their current study panel scoring processes an item that asks, “Does this study meet the requirements of the Medicare Clinical Trial Policy?”
- Approved for funding but not funded

Covered Services

Current Policy – Routine Costs

Covered outside the trial except for:

- The investigational item or service itself
- Items and services not used in the direct patient management
- Items and services customarily provided free

Includes:

- Conventional care
- Provision of the investigational item or service
- Monitoring of the effects of the item or service
- Prevention of complications
- Diagnosis or treatment of complications

New Policy

Coverable Services

- Routine Clinical Services
- Administrative Services
- Investigational Clinical Services

Routine Clinical Services

Routine clinical services include items and services that are:

- Available to Medicare beneficiaries outside of a clinical study, other than items or services that meet the definition of investigational clinical services;
- Used for patient management within the study only;
- Required solely for the provision of the investigational item or service (e.g., administration of a non-covered chemotherapeutic agent);
- Used for the clinically appropriate monitoring of the effects of the item or service (e.g., blood tests to measure tumor markers); and
- Required for the prevention, diagnosis or treatment of complications (e.g., blood levels of various parameters to measure kidney function).

Administrative Services

- All non-clinical services such as investigator salaries; protocol development; recruiting participants; data quality assurance activities, statistical analyses; dissemination of findings; and study management.
- All administrative services are noncovered.

Investigational Clinical Services

Those items and services that are being investigated as an objective within the study for its effect on health outcomes including items and services involved in providing sham procedures that are:

- Currently available to the Medicare beneficiary and thus eligible for coverage outside the trial;
- Required through the NCD process for CED and is being evaluated for its effect on health outcomes; or
- Designated by the FDA as an HUD, have received HDE status and are the investigational items or services in a study that meets the requirements of the policy.

Contact Information

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