Medicare Clinical Trial Policy: Phase I and Phase II Studies

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Cancer Clinical Trials Registered

- Search conducted of clinicaltrials.gov
- Selection criteria
  - by phase of study
  - open for enrollment
  - interventional studies
  - conducted in the U.S.
  - enrolling subjects ≥ 66 years of age
- Results (on November 21, 2008)
  - Phase I, N = 1,411
  - Phase II, N = 2,189

Background: Policy in 2000

- Experimental/Investigational items and services not covered by Medicare
- Services inside an experiment considered experimental
- 2000
  - Executive order (Clinton)
  - Established through National Coverage Determination
  - Based on 1862(a)(1)(E) of the Social Security Act
  - Agency for Healthcare Research and Quality developed criteria, allowing this section to serve as basis for policy

Background: Policy in 2007

- 2007
  - Reconsideration finalized
  - Expanded coverage through coverage with evidence development
  - Recognized coverage for items and services considered standard of care in clinical trials

Goals of the Clinical Trial Policy

- Allow Medicare beneficiaries to participate in research studies;
- Encourage the conduct of research studies that add to the knowledge base about the efficient, appropriate, effective, and cost-effective use of products and technologies in the Medicare population;
- Allow Medicare beneficiaries to receive care that may have a health benefit, but for which evidence for the effectiveness of the treatment or service is insufficient to allow for full, unrestricted coverage; and
- Limiting coverage of clinical study investigational costs to only those few studies that have the greatest likelihood of:
  - Answering questions of importance to CMS and its beneficiary population, and
  - Developing evidence that will optimize resource use.

Controversial Issues for Coverage of Phase I and Phase II Trials

- Deemed Trials
  - Qualified to meet the seven desirable characteristics.
    - Trials funded by NIH
    - Trials conducted under an IND reviewed by FDA
- Therapeutic Intent
  - One of three requirements for coverage under the policy.

Seven Desirable Characteristics

1. The principle purpose of the trial is to test whether the intervention potentially improves the patient's health outcomes;
2. 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 use;
3. The trial does not unjustifiably duplicate existing studies;
4. The trial design is appropriate to answer the research question being asked in the trial;
5. The trial is sponsored by a credible organization or individual capable of executing the proposed trial successfully;
6. The trial is in compliance with Federal regulations relating to the protection of human subjects; and
7. All aspects of the trial are conducted according to the appropriate standards of scientific integrity.

Three 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.

Medicare Coverage Policy

Trials Must Meet 2 Sets of Criteria

- Three requirements
- Seven desirable characteristics

Therapeutic Intent

Original language:

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

Therapeutic Intent

Proposed 2007 language:

The clinical research study is not designed to exclusively test toxicity or disease pathophysiology. Research studies, including some Phase I trials, whose protocols commit to measuring therapeutic outcomes as one of the objectives, may meet this standard only if the disease being studied is chronic, life threatening, or debilitating.

Therapeutic Intent

Final language in current policy:

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

Role of Medicare Contractors

Clinical Trial Policy

- Variability among contractors (FIs and Carriers)
- MMA 2003 – Medicare Contracting Reform
  - By 2009 there will be 15 Part A & B Medicare Administrative Contractors
- Decrease in variability expected

LINKS TO INFORMATION

Clinical Trial Policy

http://www.cms.hhs.gov/ClinicalTrialPolicies/

Clinical Trials Covered under Coverage with Evidence Development

http://www.cms.hhs.gov/MedicareApprovedFacilitie/

CONTACT INFORMATION

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