

# **Clinical Trials And Comparative Effectiveness**

## **The Medicare Clinical Trial Policy**

**Oncology/Hematology Carrier Advisory Committee  
Network Meeting**



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# 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 health benefit, but for which evidence for the effectiveness of the treatment or service is insufficient to allow for full, unrestricted coverage;**

**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.**



# Medicare Clinical Trial Policy: Phase I and Phase II Oncology Studies

## Controversial Issues

- **Deemed Status**
  - Qualified to meet the seven desirable characteristics of trials
    - Funded by NIH
    - Conducted under and IND
- **Three Requirements**
  - Must fall within a Medicare benefit category and not statutorily excluded from coverage
  - 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 Clinical Trial Policy: MAC Medical Directors Role

## EXAMPLE

Phase I Cancer Clinical Trial (NCI sponsored at an academic medical center)

- Medical Director denied coverage
  - Does not have therapeutic intent
- Letter to Study Site Medical Director
  - From the Presidents of the American Association for Cancer Research, Association of American Cancer Institutes, and American Society of Clinical Oncology
  - Citing the NCI Investigator Handbook and the FDA definition of “therapeutic intent” in Phase I cancer trials
- Letter from professional organizations sent to Medical Director
- Medical Director denied coverage



# The Potential for Updates to the Medicare Clinical Trial Policy

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# Comparative Effectiveness Research

**What does the CER initiative mean to the Medicare program?**

- Coverage**
- Reimbursement**
- Clinical Trial Policy**
- Coverage with Evidence Development**



# IOM Committee Definition of CER

The generation and synthesis of evidence that **compares** the benefits and harms of alternative methods to **prevent, diagnose, treat, and monitor** a clinical condition or **to improve the delivery of care**. The purpose of CER is to assist consumers, clinicians, purchasers, and policy makers **to make informed decisions** that will improve health care at both the individual and population levels.



# Great Expectations

**“At the core of both the stimulus bill and Obama’s budget is Orszag’s belief that a government empowered with research on the most effective medical treatments can, using the proper incentives, persuade doctors to become more efficient health care providers, thus saving billions of dollars. Obama is in effect betting his Presidency on Orszag’s thesis.”**

**– The New Yorker. May 4, 2009.**



# Critical Knowledge Gaps

- **The paradox**
  - 18,000 RCTs published each year
  - “Available evidence is limited or poor quality”
- **Patients, settings, comparators, outcomes, timing often not aligned with decision makers**
  - Patients, clinicians, payers, policy makers
- **Decision makers have limited traction**
  - “didn’t invite CMS because it’s a scientific meeting”



# Why so many gaps?

**The gaps, as seen by decision makers:**

- Patients are highly selected**
- Research settings are not typical of community**
- Missing or incorrect comparators**
- Physiologic or surrogate outcomes, not function**
- Results are not available when decisions made**



# Tools and Strategies for CER

- Coverage with evidence development (CED)
- Methodological guidance
- Pragmatic clinical trials (PCTs)

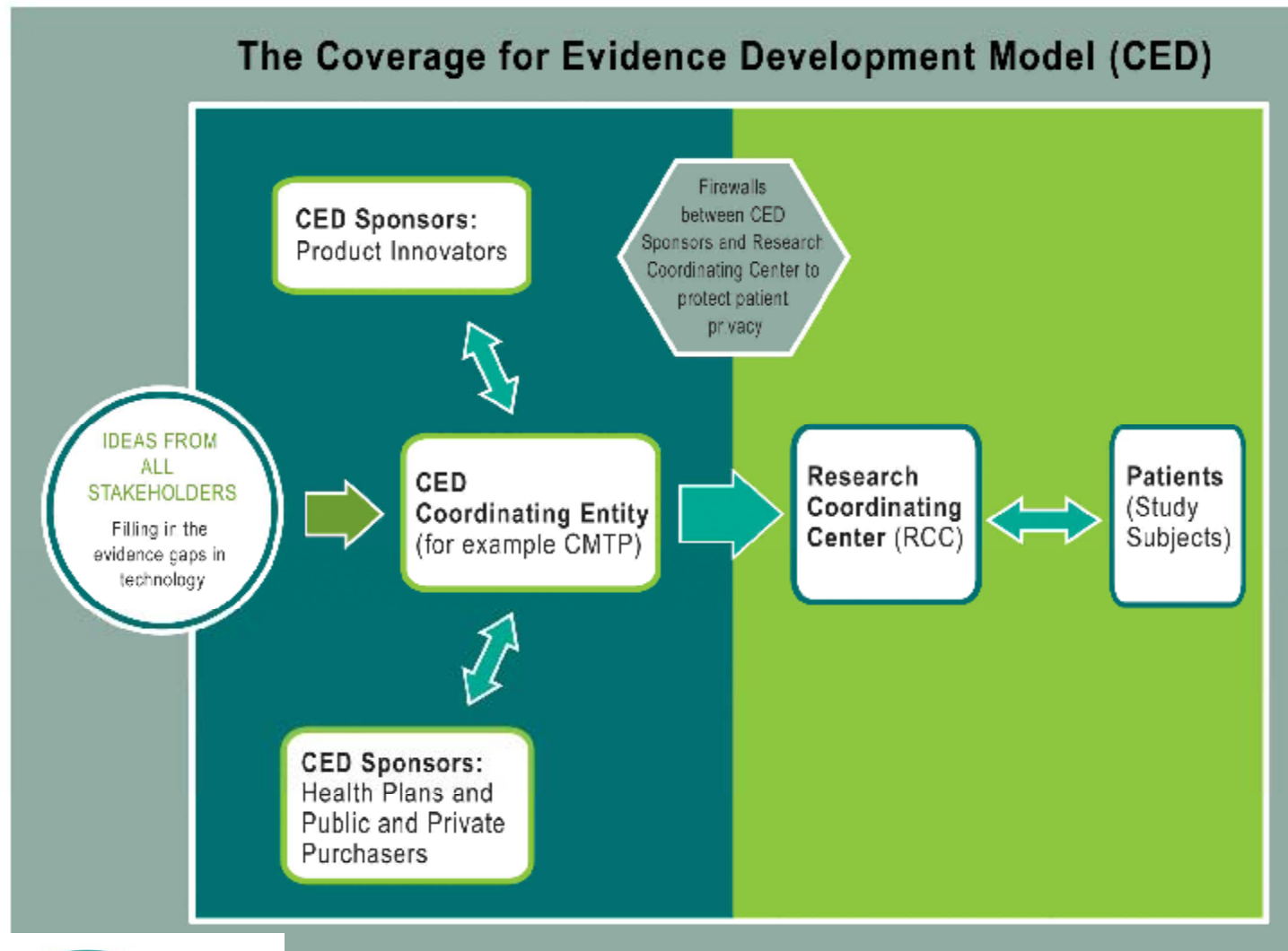


# Coverage with Evidence Development

- **Links payment to requirement for prospective data collection**
- **Intent is to guide clinical research to address questions of interest to Medicare**
  - Medicare must approve study design
- **Goal to support evidence and rapid access**
  - Lower evidence threshold with commitment to generate better info later



## The Coverage for Evidence Development Model (CED)



# Coverage with Evidence Development

## CMS National Coverage Decision (NCD) Anticancer Chemotherapy for Colorectal Cancer



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# CMTP Project Categories

IMPROVE THE QUALITY AND EFFICIENCY OF RESEARCH FOR DECISION MAKING



Trial DESIGN and IMPLEMENTATION

PRIORITIES for Evidence Development

Effectiveness GUIDANCE Documents

Applied POLICY and METHODS Projects

