Physicists on the Front Lines of Comparative Effectiveness Research

Sean Tunis MD, MSc
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Disclosure

- CMTP develops tools and strategies for comparative effectiveness research
- We receive funding from government, foundations, life sciences companies, health plans, and medical professional societies
- Aim is to serve as a neutral convener
Medicare Spending per Capita in the United States, 2003

Source: www.dartmouthatlas.org.
Calls for New “Comparative Effectiveness” Entity

- MMA Section 1013 (2003)
- Gail Wilenksy, Health Affairs (11/06)
- AHIP, BCBSA proposals (early 2007)
- MedPAC report, CBO testimony (6/07)
- Obama, Clinton, McCain health plans
- CBO final report (12/07)
- Commonwealth Fund (01/08)
- Baucus-Conrad Senate bill (07/08)
High Expectations

• “Better information about the costs and benefits of different treatment options, combined with new incentive structures reflecting the information…is essential to putting the country on a sounder long-term fiscal path.”

  • Peter Orszag, then Director of the Congressional Budget Office, June 2007
• “None of us agree on what CER actually is, but we all agree it will cost about $5B to do it”
  – Jack Rowe, former CEO, Aetna

• “Whenever you observe unanimous support for a new idea in Washington, it means that the concept has not been adequately defined”
  – Anonymous policy wonk, Washington DC
  – Jeffords variation: “Without ambiguity, there can be no unanimity.”
IOM CER Working Definition

• “The generation and synthesis of evidence that compares the effectiveness of alternative methods to prevent, diagnose, treat, monitor, and improve delivery of care for a clinical condition.
• The purpose of CER is to assist patients, clinicians, purchasers, and policy makers in making informed health decisions.”
American Recovery and Reinvestment Act
CER Funding

- $300 million to AHRQ
- $400 million to NIH
- $400 million for CER to HHS Secretary
- Vigorous organizing underway
  - IOM to report on national priorities for CER by June 2009
  - Federal Coordinating Council
  - AHRQ, NIH, AHRQ-NIH Councils
  - Blizzard of white papers
  - Battling coalitions
Medicare Review of CCTA

- EPC report from Duke (April 2006)
  - Limited evidence of clinical utility in any population
- MedCAC mtg (May 2006)
  - “Uncertain confidence about existing evidence”
- Broad local coverage of CCTA
- Medicare draft policy in 12/07 proposed CED for CCTA in “adequate” studies
- Final Medicare policy in 3/08 left local coverage in place, despite poor evidence
115 Technologies Reviewed by Kaiser

Paul Wallace, Permanente Federation

Medically appropriate

Generally not medically appropriate

Insufficient evidence because the evidence is:

A. Of insufficient quantity and/or quality
B. Conflicting or inconsistent
C. There is no evidence
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 mtg”
  – “don’t want patients messing up our protocols”
AHRQ Systematic Review: 
Tx of Clinically Localized Prostate Cancer

- Limited evidence on relative safety and effectiveness of major treatment options
  - prostatectomy, brachytherapy, radiation, active surveillance
- New technologies rapidly spreading without data
  - robotic surgery, proton beam
- Rigorous trials needed to compare treatment options, especially for side effects
Radiation for prostate cancer Compared to IMRT

Comparative Clinical Effectiveness

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Comparative Value

a  | High
b  | Reasonable/Comparative

c  | Low
From Comparative Effectiveness to Medical Policy

- **Brachytherapy Ca**: Patient information
  - Premium price
  - 0% co-pay
  - ++ Shared savings

- **IMRT**: Patient information
  - Lower reimbursed price
  - 20% co-pay

- **Proton Beam Ic**: Patient information
  - Non-coverage
  - Reference price/CED
CMTP- AHRQ- ASTRO meeting

• July 2008
  – Purpose was to discuss draft study protocol for comparative study
  – Meeting participants included:

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Methodological debate

- RCT
- Observational comparison
- Neither necessary
- Both
Key Features of the Proton/IMRT Study

- Multi-site prospective cohort study based on a registry
- Will accrue 1,600 patients (800 per arm)
- Primary outcome measures is patient reported bowel function at 2 years using EPIC
- Will also look at a number of secondary outcomes measures
Proton Beam – IMRT Status

• Study protocol completed, budget prepared
  – Observational, parallel cohort study
• R18 grant submitted in March for 2 year feasibility/pilot
  – CMTP, Outcomes, Zietman, Wallner, Konski
  – Ltrs of support from 2 IMRT and 2 proton centers
  – Ltrs of support from CMS, Aetna, United, Wellpoint
• Exploring potential collaboration for RCT with Penn, MGH, IU
Implications of CER Purpose

• If primary purpose of CER is to inform decisions
• CER requires high level of involvement of decision makers
  – Implies collaborative approach
• Future debate is not about whether outcomes studies will be needed, but about the details of the protocol
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