COMPARATIVE EFFECTIVENESS RESEARCH:

Threat or Opportunity?

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DEFINITION (1)

Comparative Effectiveness Research (CER) is:

the conduct and synthesis of systematic research comparing different interventions and strategies to prevent, diagnose, treat and monitor health conditions*

*Federal Coordinating Council for Comparative Effectiveness Research

DEFINITION (2)

… an analysis of comparative effectiveness is simply a rigorous evaluation of the impact of different options that are available for treating a given medical condition for a particular set of patients.*

* Congressional Budget Office

CER IS NOTHING NEW

- National Center for Healthcare Technology
  - Within DHHS 1978 – 1981
  - Several major studies + 75 coverage recommendations
- Office of Technology Assessment
  - Advisory agency to Congress 1979 – 1995
- Agency for Healthcare Research & Quality
  - Within DHHS 1989 – present
  - 300 staff members, $300 mm annual budget

THE CONTROVERSY IS LONG-ESTABLISHED

"The justification for most medical practices used in the United States today rests on the experience and expertise of clinicians and patients rather than on objective evidence that these practices can measurably improve people's health. Compiling objective evidence is considered by some…highly controversial, because the evidence might be applied in ways that would limit individuals' choices of medical treatments."*

* Office of Technology Assessment, 1994

CER IS INGRAINED IN REGULATORY PROCESS

- FDA review routinely demands controls
  - Optimal medical therapy
  - Established alternative devices/drugs
    - Example: LVAD for destination therapy
- CMS Coverage Analysis Group utilizes CER principles in decision making
  - NETT study of Lung Volume Reduction Surgery
  - Daily hemodialysis study
  - Focus on inclusion of > age 65 patient data

CER SEEN AS A THREAT

- The entry wedge for Cost Effectiveness Analysis?
  - Medicare effort (1989) to “back-door” limited CEA principles
- Agencies capable of interpret findings properly and subtly in making policy?
  - Fear of blanket “either-or” decisions
- Government role is a restriction on physician practice of medicine?

RECENT DEVELOPMENTS INTENSIFY INTEREST

- Need to control healthcare system costs
- Documentation of startling differences in geographic area utilization rates
- Increasing patient involvement in therapy choice
  - Web portals
- Personalized medicine revolution
  - Therapies affect sub-populations differently

ARRA 2009 CER COMMITMENT

- Federal Coordinating Council for Comparative Effectiveness Research
- $400 million allocated to DHHS for CER
  - Research
  - Human and scientific capital
  - Data infrastructure
  - Translation and adoption
- Mandated IOM review of initial priorities

IOM RECOMMENDATION: TOP CER PRIORITY AREAS

- **Health Care Delivery Systems**
- **Racial and Ethnic Disparities**
- Cardiovascular and Peripheral Vascular Disease
- **Geriatrics**
- **Functional Limitations and Disabilities**
- Neurologic Disorders
- Psychiatric Disorders
- Pediatrics

CER IN EVERY REFORM BILL

- Quality improvement
- Patient, provider and payer education
- Cost avoidance
  - Reduction in ineffective interventions
- Synergistic with mandate for electronic medical records and data exchange
- Shaped by widespread acceptance of personalized medicine principles

HOUSE BILL - HR 3962

- Center for CER within AHRQ
  - "Conduct, support and synthesize…" CER
- Independent advisory CER Commission
  - Broadly representative of affected groups
- Center for Quality Improvement w/in AHRQ
  - Best practices for quality improvement
- CMS Center for Medicare/caid Innovation
  - Test payment and service delivery models

FUNDING IN HR 3962

- 2010 – $ 90 million
- 2011 – $100 million
- 2012 – $112 million
- Beginning 2013
  - Fees on health insurance and self-insured plans
  - Formula-based “Fair share” amount per Medicare enrollee

HOUSE CER RESTRICTIONS

- Neither Center nor Commission can make coverage decisions
- Does not limit coverage of clinical trials
- Reports from Center or Commission may not be construed as mandates for coverage or payment
- No authorization for any Federal employee to interfere with the practice of medicine

SENATE BILL - REID

- Patient Centered Outcomes Research Institute
  - Independent non-profit corporation
  - Broadly representative Board of Governors
  - With mission “to assist patients, physicians, clinicians, providers and policy-makers in making informed health choices by advancing the “quality and relevance” of evidence…

SENATE FUNDING FOR CER

- 2010 - $ 10 million
- 2011 - $ 50 million
- 2012 - $150 million
- 2013-9 - $150 + fees levied on health insurance and self-insured plans
- Plus
  - 2013 - $1 per Medicare beneficiary*
  - 2014-19 - $2 per Medicare beneficiary

* 44 million in 2008 (pre-baby boom)

PCOR Institute Functions

- Identify CER priorities
- Establish research agenda
- Carry out agenda by contracts with
  - Government agencies
  - Academic research centers
  - Private organizations
  - Priority to AHRQ, NIH

AHRQ ROLE

- Disseminate CER Finds through its Office of Communication and Knowledge Transfer
- Assist users to incorporate [findings] into clinical practice
- Build CER capacity through researcher training programs
- Build data system and data management capacity

SENATE CER RESTRICTIONS

- CER results may only be used for coverage determination if –
  - Result of transparent and iterative process
  - Opportunities for stakeholder input
  - Opportunities for review and comment on proposals
  - Determinations must consider all other relevant data
  - Must consider impact on subpopulations

ADDITIONAL SAFEGUARDS

- Senate bill contains detailed requirements with regard to
  - Research methodology standards
  - Transparency
  - Integrity
  - Avoidance of conflict of interest
  - Mandatory peer review processes
  - Standards and requirements for publication

AND CER MAY NOT EVER…

- Supercede “reasonable and necessary” determinations
- Deny coverage solely based on CER
- Value wellbeing of the elderly, terminally ill or disabled lower than that of others
- Preclude individual valuation of risk/benefit tradeoff of a therapy
- Establish a $/QALY coverage standard

IF REFORM PASSES…

- Differences between House and Senate are relatively minor
- CER will contribute to some changes
  - Development of a body of publicly-funded 3rd party research on relative effectiveness of diagnostic and treatment options
  - Broader and more consistent dissemination of research findings to professionals and public
  - Increasingly well-educated and trained user community

APPROPRIATE USE OF CER FINDINGS IS LIKELY

- Legislative restrictions protect technologies from Agency over-reaction
- Strict CEA methodologies are not allowed
  - CMS will maintain current practice of more rigorous review of higher cost interventions
- Coverage determinations will increasingly consider subpopulation differences
  - But this trend is already established

CER WILL → BETTER CHOICES

- This is a threat to
  - Ineffective therapies/tests/technologies
  - Over-utilized therapies/tests/technologies
  - Those with mismatch between demonstrated utility and current utilization
  - Under-researched and unproven …
- Positive impact on health outcomes and healthcare resource utilization
  - Accretive over extended timeframe

OPPORTUNITIES FOR SOME

- Independent support for
  - Therapeutics and diagnostics that address designated high priority areas
  - Those with well-defined target treatment sub-groups
  - Those with unequivocally positive support from empirical research data
  - Currently underutilized treatment options

CER AFFECTS THE COMPETITIVE LANDSCAPE

- Levels the playing field for newer and smaller companies
  - Public funding for studies that are hard for young companies to finance
  - Data banks and registries facilitate data mining for strategy and product development
  - 3rd party research, widely disseminated, can help counter sales/marketing muscle of larger and well-established competitors

CER AND PERSONALIZED MEDICINE

- CER facilitates the right treatment for the right patient at the right time
- CER reinforces the companion diagnostics "non-blockbuster" business model
  - Segment patients by likelihood of response
  - Use segmentation to structure qualifying research and to lower development costs
  - Target utilization to achieve higher success rates and better command of smaller market

FINAL THOUGHTS

- CER does have the potential to improve outcomes and resource utilization
- The losers from a well-designed and managed CER initiative “deserve” to lose
- Resource differentials between competitors will have diminished effect
- Device and diagnostics developers will need to join Pharma in adapting to a new business and product development model

Thank You

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