Comparative Effectiveness Research: the IOM report

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Co-chair, the IOM committee for initial priority setting for CER

Background of CER

- 2003: AHRQ received $24M increase in funding →SR's, research using data sets.
- 2007-2008: Emergence of the concept of a U.S. National Program for evaluating effectiveness of health care interventions
  - Health Affairs article (Gail Wilensky)
  - IOM committee on Knowing What Works
- 2008: Endorsement by presidential candidates
- 2009: Stimulus package: $1.1B for CER

CER in the American Recovery and Reinvestment Act of 2009

- $1.1B for CER research
  - $400M to NIH
  - $300M to AHRQ
  - $400M to the Secretary, DHHS
- Mandated IOM study to establish initial priorities for conditions to study with CER funding.
  - Due date: June 30, 2009

The language of the ARRA: about the IOM

To include recommendations on the national priorities for CER to be conducted or supported with the funds provided (to the Secretary)

The Committee must consider input from stakeholders.

Source: the ARRA of 2009

Committee Members

- Christine Cassel
- Kay Dickersin
- Alan Garber
- Constantine Gatsonis
- Gary Gottlieb
- Sheldon Greenfield
- James Guest
- Mark Helfand
- Carolina Hinastrosa
- George Isham
- Arthur Levin
- JoAnn Manson
- Katie Maslow
- Mark McClellan
- Sally Morton
- Neil Powe
- Joe Selby
- Lisa Simpson
- Harold Sox
- Sean Tunis
- Steven Udvarhelyi
- Eugene Washington
- James Weinstein

The IOM Committee's working 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.

Source: iom.edu/cerpriorities

What's unique about CER?

- Direct, head-to-head comparisons.
- Broad range of topics.
  - tests, treatments, strategies for prevention, care delivery and monitoring
- Focus on patient-centered decision-making
  - tailor the test or treatment to the specific characteristics of the patient.
- a broad range of beneficiaries:
  - patients, clinicians, purchasers, and policy makers.
- Study populations representative of clinical practice

“Patient-centered”

- Suppose a RCT shows that A>B, but many patients got better on B.
  - Lacking any additional knowledge, you should prefer B.
- Is it possible that some patients would have done better on B than A?
  - Can we identify them in advance?

The Promise of CER

Information to help doctors and patients make better decisions

Stakeholder input

- March 20th open meeting at NAS building
  - 56 presenters
- Web-based survey open to anyone
  - Asked for 3 condition-intervention pairs in order of priority
  - ~1758 unique respondents
  - ~2606 nominations

Priority-setting criteria

- morbidity and mortality
- cost
- funding gap (e.g., minimal research is being done)
- Research gap
- potential to act on the information once generated
- disease burden
- controversy

Source: iom.edu/cerpriorities

Other information requested

- Category of condition (e.g., skin disorder)
- Study population

- Type of intervention
- Type of research design

Source: iom.edu/cerpriorities

Portfolio Criteria

- The committee should develop a balanced portfolio of topics for CER research.
  - i.e., avoid having all the topics be on heart disease or for adults
- Criteria: population affected, condition category

IOM Committee's Voting Process

2,606 recommended CER topics received from 1758 respondents to web-based questionnaire

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Round1 Voting = 1,268 nominated topics→ 200 topics

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Round 2 Voting = 145 rank-ordered topics

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Committee discusses each topic
Round 3 Voting on 155 nominated topics

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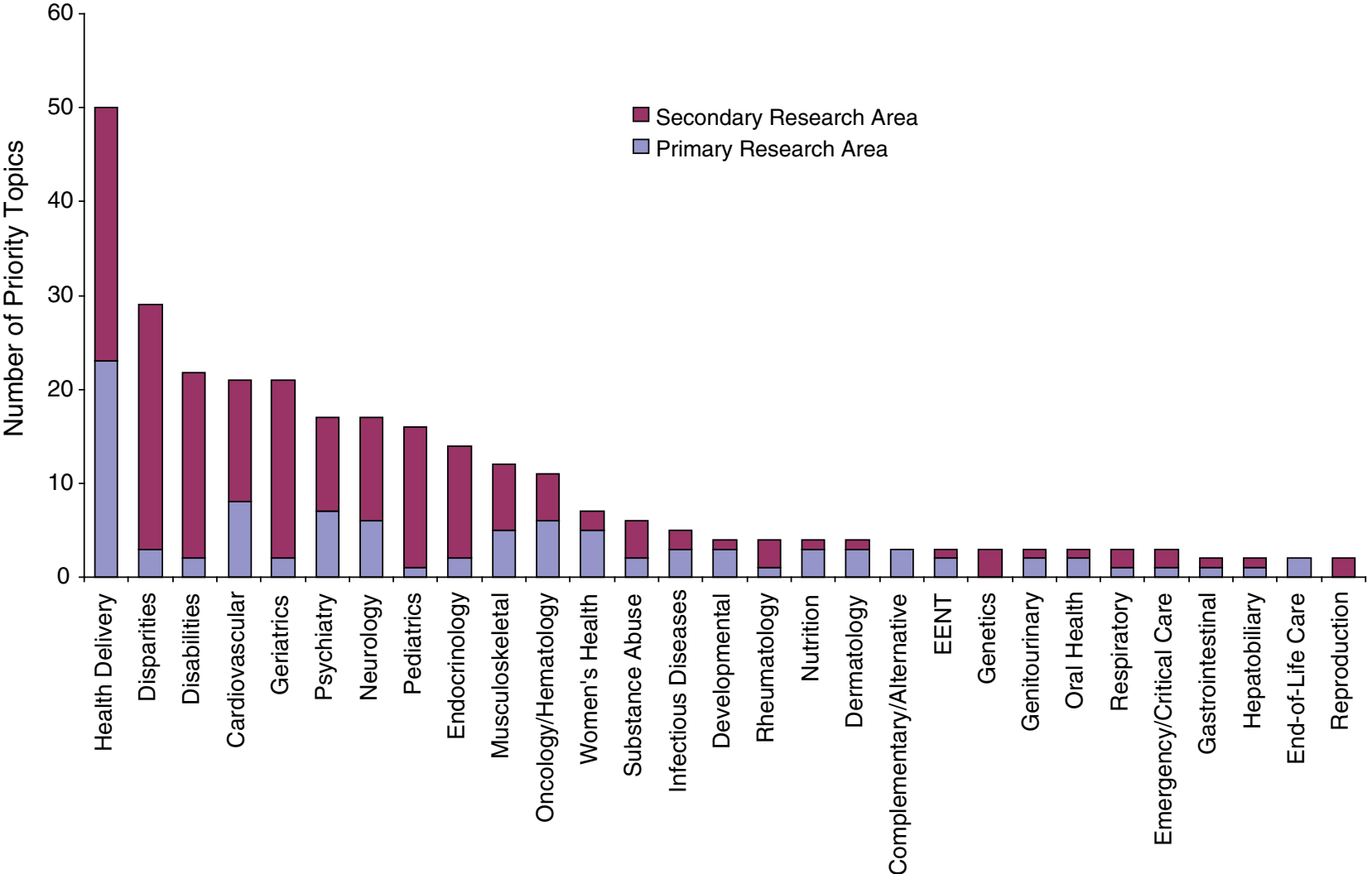
Round 3 Results = Final 100 priority topics

Figure 5.1 Distribution of the recommended research priorities by primary and secondary research areas



Sample top quartile topics

- Compare the effectiveness of treatment strategies for atrial fibrillation including surgery, catheter ablation, and pharmacologic treatment.
- Compare the effectiveness of the different treatments (e.g., assistive listening devices, cochlear implants, electric-acoustic devices, habilitation and rehabilitation methods [auditory/oral, sign language, and total communication]) for hearing loss in children and adults, especially individuals with diverse cultural, language, medical, and developmental backgrounds.

Sample top quartile topics

- Compare the effectiveness of primary prevention methods, such as exercise and balance training, versus clinical treatments in preventing falls in older adults at varying degrees of risk..
- Compare the effectiveness of upper endoscopy utilization and frequency for patients with gastroesophageal reflux disease on morbidity, quality of life, and diagnosis of esophageal adenocarcinoma.

Sample top quartile topics

- Compare the effectiveness of dissemination and translation techniques to facilitate the use of CER by patients, clinicians, payers, and others.
- Compare the effectiveness of comprehensive care coordination programs, such as the medical home, and usual care in managing children and adults with severe chronic disease, especially in populations with known health disparities.
- Compare the effectiveness of different strategies of introducing biologics into the treatment algorithm for inflammatory diseases, including Crohn's disease, ulcerative colitis, rheumatoid arthritis, and psoriatic arthritis.

Sample top quartile topics

- Compare the effectiveness of various screening, prophylaxis, and treatment interventions in eradicating methicillin resistant *Staphylococcus aureus* (MRSA) in communities, institutions, and hospitals.
- Compare the effectiveness of strategies for reducing health care associated infections (HAI), including catheter-associated bloodstream infection, ventilator associated pneumonia, and surgical site infections in children and adults.
- Compare the effectiveness of management strategies for localized prostate cancer (e.g., active surveillance, radical prostatectomy [conventional, robotic, and laparoscopic], and radiotherapy [conformal, brachytherapy, proton-beam, and intensity-modulated radiotherapy]) on survival, recurrence, side effects, quality of life, and costs.

The IOM: the CER program should also:

- Do priority-setting on an ongoing basis.
- Have a broadly representative oversight committee
- Engage public participation at all levels of CER
- Support large-scale, clinical and administrative data networks
- Do research on dissemination of CER findings
- Support research and innovation in the methods of CER
- Expand and support the CER workforce

CER Institute Legislation

- The Senate Finance Committee white paper: Call to Action: Health Reform 2009
  - Private, non-profit (avoid political interference)
  - Governing board a mix of federal and private sector
  - Would contract with public agencies and private sector.
- House bill (2009)
  - Government-based (AHRQ)
  - Much detail about the oversight committee

Expectations and CER

- No one knows how to control future health care cost inflation.
  - People do believe that better decisions could help to control future costs.

- Everyone realizes that CER is not a quick fix to the runaway cost problem.
  - On a 10 year time-horizon, the Congress does expect research to improve decision-making and perhaps reduce costs.

Everyone has an interest in seeing CER succeed

How can I help?

Helping CER to succeed

- Make sure that CER funds are used to support CER, with its focus on better decision making, typical study populations, and head-to-head comparisons.
  - Funding agencies
  - Researchers
  - Study sections
  - The public: by holding funding agencies accountable
- Hold authors to high standards of research practice: a role—and a challenge--for journals.

CER methodological challenges to researchers and journals

- Focus on decision making
  - The scope of CER should include research on all aspects of decision making by doctors and patients.
- Less transparent methods:
  - Adaptive trials
  - More complex modeling (systems biology)
- Observational research on huge data sets taken from the records of actual patient care:
  - missing data
  - missing outcomes
  - unmeasured confounders

CER methodological challenges

- Trying to find the clinical predictors of response to therapies.
  - Helps to individualize decision making.
  - Role of cross-over trials
- Trying to measure the marginal value of information
  - predicting present disease status (sensitivity, specificity, post-test probability)
  - future outcomes (reclassification tables)

CER methodological challenges

- Adherence to all but the simplest guidelines is relatively poor.

- We need to identify interventions that improve adherence to good practice
  - A high priority topic for the IOM committee
  - Often require study designs that are not as definitive as randomized trials.

Questions for the future

- Will Congress enact a national CER program?
- What will it do? Systematic reviews, fund original research, guidelines?
- Will the Secretary take guidance from the IOM committee? Will funding agencies?
- Will doctors use the results of CER?
- Will patients play a role in decision making?