Knowing What Works in Health Care: A Roadmap for the Nation
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IOM Committee on Reviewing Evidence to Identify Highly Effective Clinical Services
Committee Members

- Barbara McNeil
- Harold Sox
- Allen Daniels
- Kay Dickersin
- Jill Eden (IOM staff)
- Robert Galvin
- Dana Goldman
- Richard Justman
- Arthur Levin
- Richard Marshall
- Wilhelmine Miller
- Sally Morton
- Samuel Nussbaum
- Diana Petitti
- Steven Shak
- Lisa Simpson
- Glenn Steele
- Ben Wheatley (IOM staff)
Charge to the Committee

• Robert Wood Johnson Foundation (June 2006)
  - Recommend an approach to identifying highly effective clinical services
  - Recommend a process to evaluate and report on clinical effectiveness
  - Recommend an organizational framework for using evidence reports to develop recommendations on appropriate clinical applications for specified populations
An Imperative for Change

- Constraining health care costs
- Reducing geographic variation in the use of health care services
- Improving quality
- Consumer-directed health care
- Making health coverage decisions
Committee Recommendations

- Congress direct Secretary of HHS to designate a single entity to ensure production of credible, unbiased information about what is known and not known about clinical effectiveness.
- Secretary of HHS should appoint a Clinical Effectiveness Advisory Board to oversee the Program.
- Develop standards to minimize bias for priority setting, evidence assessment and recommendations development.
- Appoint a Priority Setting Advisory Committee to identify high priority topics
- Develop evidence-based methodologic standards for systematic reviews, including a common language for characterizing the strength of evidence
- Assess the capacity of the research workforce to meet the Program’s needs if necessary expand training opportunities in systematic review and comparative effectiveness research methods.
- Groups developing clinical guidelines should use the Program’s standards.
- Minimize bias: balance of competing interests, publish conflict of interest disclosures, prohibit voting by members with material conflicts
- Stakeholders should preferentially use clinical recommendations according to the Program standards.
Conceptual Framework

- Research studies
  - Randomized controlled trials
  - Cohort studies
  - Case control studies
    - **Case-control** studies identify factors that may contribute to a medical condition by comparing a group of patients who have that condition with a group of patients who do not.
  - Cross-sectional studies
    - **Cross-sectional studies** involve observation of some subset of a population of items all at the same time, in which, groups can be compared at different ages with respect of independent variables. Cross-sectional studies take place at a single point in time in contrast to longitudinal studies which include measurements taken over a period of time. Cross-sectional research assumes that the attributes of the targeted group are typical of the whole group.
  - Case series

- Systematic review
  - Identify and assess the quality of individual studies
  - Critically appraise the body of evidence
  - Develop qualitative or quantitative synthesis

- Clinical guidelines and recommendations
Systematic Review

- Characteristics of the patient population, care setting and type of provider
- Intervention (route, dose, timing, duration)
- Comparison group
- Outcome measures and timing of assessments
- Quality of the evidence, i.e., risk of bias
- Sample sizes
- Quantitative results and analyses including examination of whether the study estimates of effect are consistent across studies
- Effect of potential sources of study heterogeneity if relevant
UnitedHealthcare Perspective: Hierarchy of Evidence

β Statistically robust, well-designed randomized controlled trials
β Statistically robust, well-designed cohort studies
β Large, multi-site observational studies
β Single-site observational studies
β In the absence of incontrovertible scientific evidence, medical policies may be based upon national consensus statements by recognized authorities.
β Expert opinion
Rules of Engagement

- No health service will be deemed unproven solely on the basis of a lack of randomized controlled trials.
- We will develop no medical policies based solely on expert opinion.
- Professional specialty societies will be engaged in the development of medical policies.
  - to actively solicit comment on areas of a medical policy where a gap in clinical evidence has been identified and expert guidance is requested
  - to offer the specialty society the opportunity to comment on any other portion of the policy if they so desire. UnitedHealthcare will always attempt to be transparent in identifying potential areas of controversy. However, specialty societies will also be free to identify particular areas on which they wish to comment.
- Guidance from external sources notwithstanding, UnitedHealth Group and UnitedHealthcare reserve the right and have ultimate responsibility to develop and implement medical policies and to use their conclusions to make benefit coverage decisions.
Emerging Promising Treatments

- Rare diseases or conditions
- Treatments for conditions with no known effective treatment
- Comparative Effectiveness trial unlikely to be completed in the short term
- Clinical Trials
- “Coverage with Evidence Development”
- “Protocol-Specified Prospective Data Collection” (PSPD) programs
Examples of Promising Technologies

- Accelerated partial breast irradiation
- Bevacizumab to treat age-related wet macular degeneration
- Endovascular repair of thoracic aortic aneurysm
- Multigene assays for treatment planning for breast cancer
- Wingspan® intracerebral stent for prevention of stroke
- Endobronchial valves for lung volume reduction
- Bronchial thermoplasty to treat moderate-to-severe asthma
- Injectable bulking agents to treat vesicoureteral reflux in children
- Intravenous ferumoxytol as an alternative to erythropoietins in persons with chronic renal failure
• Questions??