Evidence-based Decision Making

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Conclusions

- Clinicians and patients / consumers make decisions every day, whether they have information or not.
- **Opportunity**: to provide the best available information, even if it is not definitive, when needed, in a way they can understand.
- Evidence is dynamic - we should expect it to change so we must revisit on a regular basis.
- Transparency is critical.
- Enhanced evaluation should not stifle innovation.
- Continued collaboration between public and private sectors is essential.
Overview

- The Challenge: Creating Actionable Knowledge for Decision Making
- AHRQ’s Effectiveness Program
- Future Opportunities
Treatments Thought to Work but Shown Ineffective

- Sulphuric acid for scurvy
- Leeches for almost anything
- Insulin for schizophrenia
- Vitamin K for myocardial infarction
- HRT to prevent cardiovascular disease
- Flecainide for ventricular tachycardia
- Routine blood tests prior to surgery
- ABMT for late stage Breast CA

## Diffusion of knowledge

<table>
<thead>
<tr>
<th>Clinical Procedure</th>
<th>Landmark Trial</th>
<th>Current rate of use</th>
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<tbody>
<tr>
<td>Flu Vaccine</td>
<td>1968</td>
<td>64% (2000)</td>
</tr>
<tr>
<td>Pneumococcal Vaccine</td>
<td>1977</td>
<td>53% (2000)</td>
</tr>
<tr>
<td>Mammography</td>
<td>1982</td>
<td>75.5% (2001)</td>
</tr>
<tr>
<td>Cholesterol Screening</td>
<td>1984</td>
<td>69.1% (1999)</td>
</tr>
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Root Causes

- **Clinical uncertainty**
  - Practice variations persist
  - Delayed or obsolete data from clinical studies
  - Few tools for customizing results of clinical trials to individual patients
  - Under-developed dissemination infrastructure

- **Rising costs**
  - Blunt containment – or informed decisions
  - Proliferation of new diagnostic and treatment options
  - Underuse, overuse and misuse
WE HAVE TWO OPTIONS.
EITHER AN EVIDENCE-BASED TREATMENT OR AN EXCITING, RISKY ALTERNATIVE.
Uses of Evidence for Decisionmaking

- Product approval
- *Product purchasing and formulary selection*
- *Benefit and coverage decisions*
- Practice guidelines
- Quality review and improvement
- *Clinician/patient decisionmaking*
- Choosing plans or providers
- Organizational and management decisions
- Program financing and priority setting
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AHRQ Mission Statement

To improve the quality, safety, efficiency, and effectiveness of health care for all Americans
AHRQ focuses on the Health Care System

- Assess the *effectiveness*, *comparative effectiveness*, and *cost-effectiveness* of health care services.
- Identify ways to improve *patient safety* and *quality* of health care systems.
- Advance the appropriate use of *health information technology*.
- Understand *system issues*: role of organizational design, management, workflow, management, and incentives on efficiency and effectiveness.
- Develop *data* on the health care system for monitoring and decision-making.
AHRQ: Strategic Redirection

Accelerating the Pace of Innovation

- **Ensuring Value** through More Informed Choice
- **Assessing** Innovation **Faster**
- **Implementing** Effective Interventions **Sooner**
Working Definitions

Effectiveness
- Can it work?
- Does it work in practice?
- Is it worth it?

Comparative Effectiveness
- Comparison of the effectiveness of various treatments and procedures - looking at which treatments for specific clinical problems work best for whom

What is Section 1013?

- To improve the quality, effectiveness and efficiency of health care delivered through Medicare, Medicaid and the S-CHIP programs.
- $15 million is appropriated in Fiscal Year 2005 for the Agency for Healthcare Research and Quality (AHRQ) to conduct and support research with a focus on outcomes, comparative clinical effectiveness and appropriateness of health care items and services (including pharmaceutical drugs), including strategies for how these items and services are organized, managed and delivered.
What is Section 1013?

- By June 2004, the Secretary shall establish an initial list of research priorities (including those related to prescription drugs).
- Priorities may include health care items and services which impose a high cost on Medicare, Medicaid or S-CHIP, including those that may be underutilized or overutilized.
What’s Different?

- Validation of systematic approach to evaluating and communicating effectiveness

- A total program from synthesizing knowledge, generating knowledge, communicating knowledge

- Transparent
Legs of the Program

Evidence Synthesis
Evidence Generation
Evidence Communication
Staging Considerations

- Initial effort must focus on evaluating and synthesizing **available data** related to the identified priorities (AHRQ designated)
- Studies to generate **new knowledge** needed quickly (statute does not designate agency to conduct these studies)
- Broad and **sector-relevant dissemination** mandatory (AHRQ designated)
Priority List: Initial Issues Considered

- How should the lists be prioritized?
  - Level of uncertainty
  - Burden of illness
  - Proportion of population affected
  - ???

- What should the framework look like?
  - Condition
  - Intervention
  - ######
Office of the Secretary designated an inter-agency Steering Committee to guide priority-setting under section 1013.

HHS staff with expertise in following areas consulted: clinical practice, health services research, epidemiology, research design and synthesis, policy evaluation, outcomes evaluation, economic evaluation, technology assessment, and pharmaceutical utilization and outcomes.
The Process for Development of Prioritization Criteria (2)

- Reviewed relevant prioritization evaluations
  - AHRQ prioritization for technology assessments
  - Institute of Medicine recommendations for guideline development
  - NIH prioritization of research topics
  - OMAR criteria for topic selection for consensus documents
  - USPSTF criteria for prevention topics
  - Selected non-Federal efforts
What is Being Prioritized?

- Committee staff considered several approaches, including:
  - Prioritize specific conditions for evaluation, e.g. diabetes, asthma, cardiovascular disease
  - Prioritize specific interventions for evaluation, e.g. specific drugs or drug classes
  - Prioritize specific clinical intervention strategies, e.g. use of cholesterol lowering drugs for primary prevention of cardiovascular disease
Five Criteria Chosen

- Prevalence of a condition
- Burden of a condition
- Cost of care of a condition
- Disproportionate representation of the condition in the Medicare population
- Potential for impact

Note: First four criteria used specifically for identifying priority conditions. Since potential for improvement is heavily dependent on the specific clinical strategy, this was used to consider the clinical intervention strategies for a particular condition.
Stakeholder Involvement

- Call for written suggestions from public and private sectors
- Listening session May 21
- Approximately 300 comments received
- Two meetings of the DHHS Steering Committee held
- Concluded on-going involvement of stakeholders necessary to assure relevance of research
Development of a Framework

- **First stage of prioritization:**
  - specific conditions chosen based upon those that have the largest impact on stakeholders using prioritization criteria

- **Second stage:**
  - within each condition, specific clinical strategies are prioritized

- **Rationale:**
  - decision makers can evaluate the priority clinical areas to determine the amount of funding that should be devoted to each clinical condition
  - interventions needed to be evaluated within the context of specific conditions and clinical strategies
Example

Condition X

- What is the effectiveness of Device Y in diagnosing Condition X in community settings?
- What is the comparative effectiveness of Pharmaceutical C vs. Pharmaceutical E in treating Condition X in ambulatory settings?
- What are the barriers to implementing Intervention E in the management of Condition X?
Priority List

- Ischemic Heart Disease
- Cancer
- Chronic Obstructive Pulmonary Disease/Asthma
- Stroke, Including Control Of Hypertension
- Arthritis And Non-traumatic Joint Disorders
- Diabetes Mellitus
- Dementia, Including Alzheimer's Disease
- Pneumonia
- Peptic Ulcer/Dyspepsia
- Depression And Other Mood Disorders
Centers for evidence synthesis and review

- 13 Evidence-based Practice Centers
- Others?
- Evidence syntheses on effectiveness and comparative effectiveness
- Identify and detail research gaps
Infrastructure (2)

- Resource Center for Effectiveness Research
  - Methodology resource
  - Stakeholder input
  - Dossier process
  - Formative help
  - Research coordination
Infrastructure (3)

- Center for Decision Support
  - Systematic Review translation for different levels of decision makers
  - Translation into different mediums
  - Medical decision support generally – how to interpret acceptable risk, relative risk, rates, etc.
Rapid turnaround research centers
  - Capitalizing on data
  - New methods
  - Answers for questions that don’t require multi-year, multi-million $ trials
Continued Input

- Web casts, listening sessions, invited comments from public and private sectors on:
  - Priority lists and questions
  - Format and dissemination of the reports
- Close coordination with CMS
- Meetings of Steering Committee
Dissemination and Translation

- Broad and creative
- Tailored to different audiences
- Use different mediums, electronic, paper, other media
- Keep current
Approaching Knowledge Gaps

- Short turnaround research capacity
  - Not always head to head
  - Need to be creative
  - Explore new methodologies
  - Examine existing or forthcoming data sources
  - Reserve most expensive approaches for the most important and controversial questions
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Types of Decisions

- Public policy
  
  *Do we fund heart transplants?*

- Systems policy

  *Where do we “locate” heart transplant services?*

- Clinical policy ("EBM")

  *Who should receive heart transplants?*
Important Opportunities

- Common information infrastructure for care improvement and surveillance, observational studies, registries?
- Strategy for rapid response to decision makers’ needs for information
- Practical clinical trials
- Link to maintenance of certification
- Rapid-cycle evaluation that keeps up with technological innovation
- Can we address quality (doing it well) and comparative effectiveness concurrently?
Improving the quality of clinical research will depend on more active involvement of clinical and health policy decision makers in all aspects of clinical research, including priority setting, study design, study implementation, and funding.
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