Comparative Effectiveness: Better Care or Rationing?

Nancy Davenport-Ennis
President & CEO
National Patient Advocate Foundation

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“The nation’s approach to the performance of CER must be structured to ensure continuous learning and the rapid translation of the best available evidence into clinical practice.”

- “Improving Medical Decisions Through Comparative Effectiveness Research: Cancer as a Case Study”
History of Comparative Effectiveness Research (CER)

Agency for Healthcare Quality & Research (AHRQ)
- Launched initiative in 1997 to promote evidence-based practice in everyday care through establishment of 12 Evidence-based Practice Centers (EPCs).
- EPCs develop evidence reports and technology assessments on topics relevant to clinical, social science/behavioral, economic, and other health care organization and delivery issues – specifically those that are common, expensive, and/or significant for the Medicare and Medicaid populations.
- With this program, AHRQ became a “science partner” with private and public organizations in their efforts to improve the quality, effectiveness, and appropriateness of health care by synthesizing the evidence and facilitating the translation of evidence-based research findings.

Centers for Medicare & Medicaid Services (CMS)
- The document outlined the circumstances under which CMS would issue a national coverage determination (NCD) requiring, as a condition of coverage, collection of additional patient data to supplement standard claims data.

European Experience

Following the early examples of Australia and Canada, the United Kingdom established the National Institute for Health and Clinical Excellence (NICE) in April 1999 and since then, its appraisals have attracted international attention.

The UK’s NICE has re-opened consultation on the availability of drugs to treat myeloma and kidney cancer, which were previously rejected on cost grounds.

The reversal follows NICE’s initiative to relax cost-effectiveness criteria under certain circumstances when patients are close to death and concessions on costs by manufacturers.
NPAF Activities on CER

- Developed CER principles – January 2008
  - Available at [www.npaf.org](http://www.npaf.org)

  - Letter citing concerns that (1) patient representatives were left out of process/Federal Coordinating Council and (2) House report language implied that CER would be used to make cost-effectiveness decisions

- NPAF President & CEO testified before Federal Coordinating Council on CER – May 2009

- NPAF Policy Consortium meeting on CER – May 2009

- “Improving Medical Decisions Through Comparative Effectiveness Research: Cancer as a Case Study” – May 2009
  - Released by Friends of Cancer Research and twenty-five leading patient advocacy organizations, including NPAF
  - Includes four recommendations for the expansion of comparative effectiveness research for policy makers and government officials
  - Available at [www.npaf.org](http://www.npaf.org) or [www.focr.org](http://www.focr.org)

NPAF Principles on CER

- Support the development of research on the comparative clinical effectiveness of multiple treatment options

- Purpose of CER should be to improve the quality, safety and delivery of care, not to limit access, deny treatment or reimbursement

- CER must support and incent the development of personalized medicine
NPAF Principles on CER cont..

- Research should encompass all aspects of medical treatment and *special patient populations, including minorities, should be integrated* into comparative effectiveness research.

- A process should be in place to collect financial/cost information that can be used to *inform* decision-making between physicians and patients.

- Legislation must ensure that newly approved drugs will be immediately available for use by patients.

Improving Medical Decisions Report

Recommendations

Expanding CER in the U.S. is essential to provide reliable data on the risks and benefits of health interventions, so that this information can be used by patients and physicians, professional medical societies developing practice guidelines or clinical recommendations, public and private health care purchasers, and other health care decision-makers.

**Recommendations #1:** A comprehensive CER program should be developed to better identify the most effective health care options.

**Recommendation #2:** The program should link data from public and private entities to build upon existing data collection and research capabilities.
Recommendation #3: CER studies should support the development of “personalized” or stratified medicine

- Example: KRAS gene in colorectal cancer patients
- Retrospective analyses of data from several large clinical studies showed that patients with metastatic colorectal cancer who had the normal KRAS gene had improved survival outcomes when treated with Erbitux® or Vectibix® rather than with standard chemotherapy alone, but that patients with a mutant, activating form of the KRAS gene did not benefit.
- Determining whether colorectal cancer patients have a normal or mutant form of the KRAS gene is essential, so that treatment can be targeted to the appropriate subset of patients.

Recommendation #4: Processes should be developed to ensure that information gained through CER is incorporated into clinical practice and better informs decisions made among patients, their health care providers, and payers.
“Studies should aim to reduce health disparities and close the gap between the care that we already know works well and the care patients actually receive.”