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Today's Program



- **Presenter**
 - David B. Nash, MD, MBA

- **Moderator**
 - Stan Heimberger, PhD, MBA

- **Q&A follows the presentation—but feel free to send in questions as you have them**

HEOR 101

Comparative Effectiveness Research: Ration or Rational

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Objectives

- Define Comparative Effectiveness Research (CER)
- Discuss the Need for CER
- Evaluate the Implications of CER
- Provide Insight to the Future of CER

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Comparative Effectiveness Research

- Several definitions of CER, but the underlying concept is the same
 - Congressional Budget Office: a rigorous evaluation of the impact of different options that are available for treating a given medical condition for a particular set of patients¹
 - Institute of Medicine: 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.²

Characteristics of CER

- Allows for the direct comparison of effective interventions for a particular disease state
- Research involves evaluating “real world” data
- Aimed at assisting clinicians make better decisions based on the needs of individual patients³
- Cost is NOT a component of CER¹

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The Need for CER

- Large discrepancy between health care spending and outcomes observed globally and nationally⁴
- CER can help answer the question, “Why are we spending so much more money and not seeing an improvement in outcomes?”

Global Perspective

- According to the World Health Organization (WHO) The US spends 15.3% of its GDP of healthcare, more than any other industrialized nation, but its citizens are not the healthiest.⁵

Country	Life Expectancy	IMR per 1000	% low birth weight	Health spending as %GDP
Australia	82	5	7	9.2
Canada	81	5	6	10.1
UK	79	5	8	8.3
USA	78	7	8	15.3

National Perspective

- Medicare spending per enrollee by state, 2006
- After accounting for geographical location, greater gains in survival were NOT observed with increased costs⁴

STATE	SPENDING
Seattle, WA	\$7,218
Philadelphia, PA	\$9,665
Manhattan, NY	\$12, 114

Lack of Consensus

- Discrepancy between spending and outcomes can be attributed to the lack of consensus between the effectiveness of various treatment options
- 135 physicians were each asked to treat the same patient, the study resulted in 82 different treatment options
- Within the same hospital, the patterns of care for a given medical condition can vary by 80%⁶

Variation of Healthcare

- With the vast number of treatment options for any given condition, it comes as no surprise that there is such variation in practice patterns
- Very little of that variation is based on medical science or efforts to improve the quality of care
- Very few standards exist relative to establishing the comparative value of new drugs and devices⁶

Lack of Information

- Clinical trials often compare new drugs to placebo
- Without head-to-head trials between the new drug and the current standard, the true advantage of one therapy over another is unknown
- Failure of the drug development and approval process to provide data that can assist clinicians in choosing the most appropriate care¹

Efficacy versus Effectiveness

- Efficacy is often derived from randomized controlled studies
 - Strict inclusion/exclusion criteria
 - New drug vs. placebo
 - “What a drug can do” in a controlled setting
- Effectiveness is a measure of what a drug will do
 - New drug vs. comparator
 - Evaluates drug use in the real world population
 - Ability to detect rare adverse events not found during clinical trials¹

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The Role of CER in Healthcare

- CER can provide clinicians and health plans with the ability to compare treatments to each other, providing valuable information in assisting with clinical decisions
- CER will provide valuable effectiveness data derived from the real world setting³
- Generate effectiveness data in under-represented populations, i.e., children, elderly, and minority groups²

The Status Quo

- Growing uncertainty about the standard of care involving treatments and technologies
- Great disparity of information between clinicians and patients
- Unexplained variations in healthcare and costs across the US
- There is a growing need to justify the uncontrolled growth in healthcare spending⁷

Institute of Medicine

- Given the task of generating a list of high priority research questions for CER to address
- IOM committee made over 20,000 solicitations and received input from direct mail, a public session, and a web-based questionnaire²

IOM Topics

- Topics were chosen to address broad societal needs
- Identified diseases and conditions with the greatest overall effect on the health of the US population
- Focus on less common conditions that affect individuals in under-represented subgroups
- Include interventions such as disease prevention, systems of care, drug therapies, devices, surgery, and monitoring of disease²

Prioritizing Research Topics

- IOM committee developed a criteria for assessing the importance of research topics
- Criteria included: cost and burden of disease, appropriateness and applicability of CER, and the likelihood that CER would result in improved outcomes
- After three rounds of voting, the list of topics were narrowed to 100²

Potential Impact of CER

- Shift the responsibility of CER from the government to the manufacturer
 - Although drug vs. placebo trials are appropriate for FDA approval, head-to-head comparator trials are necessary to accurately assess a drug's relative effectiveness
 - Superiority claims should be fully supported by data collected by the manufacturer⁸
- Provide a wealth of information to assist clinicians in making sound evidence-based decisions

Improvement of Care

- CER will not dictate clinicians' practice methods, it is designed to systematically evaluate available treatment options to assist clinicians in evaluating best practices
- Promote adherence to best practices and improve the quality of care in disparate regions
- Make it easier for clinicians to gauge a particular therapy's place within a treatment algorithm¹

CER and Cost

- Reduce the utilization of treatments that cost more, but do not result in improved outcomes
- CER can highlight areas of opportunity to reduce costs while improving quality
- Provide an explanation why higher per capita spending on healthcare does not result in better outcomes

Spark Innovation

- Push the pharmaceutical industry to create new drugs that are truly innovative that result in significant improvement in patient outcomes
- Shift away from “me-too” drugs that cost more, but provide little to no additional benefit
- Push manufacturers to develop medical devices that are superior to the current standard of treatment and have sufficient evidence to support these claims

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Opposition to CER

- Issues with CER being sponsored by the government
 - Viewed as an attempt to ration care
 - Seen as a component of nationalized health care
- Some believe that CER will eventually determine what the government is willing pay for healthcare
- Including cost into CER, or cost-effectiveness analysis, may limit access to more expensive treatments⁹

Was Any of That True?

- The strong notion that CER was linked with a single-payer system has resulted in political action stating that CER will NOT be used to determine payment policy²
- CER will not leading rationing of care, but will result in rational decision making
- Contrary to popular belief, cost-effectiveness analysis often justifies the use of more expensive treatments due to better outcomes achieved³

The Future of CER

- CER is already being used in the UK and Canada
 - Has led to cost savings while maintaining or improving the quality of care¹
 - The health system in these countries vary from that of the US, but the concept and insight of CER remains the same
- CER is a step towards removing healthcare discrepancy across the US by providing physicians with a tool to evaluate best care practices

Sorting Out Healthcare

- The multitude of health interventions available for any given medical condition has led to confusion and disparity
- CER is a scientifically-based method to untangle the knotted web of healthcare interventions
- For a given condition, CER will provide a structured list of healthcare interventions organized by their outcomes observed in a real world population

Moving Forward

- CER will serve as a great source of knowledge for both clinicians and patients
- Excellent tool to assist clinicians with making treatment decisions based on sound evidence
- Although the government is initiating CER, the responsibility should shift to manufacturers
- Promote true innovation, where “me-too” drugs are no longer good enough
- Drugs would need to prove their value

Closing Remarks

- CER is not meant to dictate physician practice
- CER is not meant to ration care
- CER is not a segue to socialized medicine
- At its core, CER is designed to improve the quality of care
- CER is not based on cost, but true outcomes important to physicians and patients

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Questions & Answers

To ask a question, please type your query into the 'Q&A' chat box at the bottom left of your screen. Every attempt will be made to answer all questions.

Next ISMPP U



DATE: November 18th—second of a special two-part program focusing on health economics outcomes research

TIME: 11am EST—NOTE NEW TIME

TOPIC: HEOR 201- How Valuable is this Medical Technology? The Role of HEOR

Thank you for attending!

We hope you enjoyed today's presentation.

Please take a moment to offer your valuable feedback, as it will help us to develop future educational offerings.

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