# *Promises and Challenges of Shared Decision Making*

## National Coalition for Cancer Survivorship & IOM National Cancer Policy Forum Workshop

**Sheldon Greenfield, MD**
*Health Policy Research Institute*
University of California, Irvine
February 28, 2011

# *Goals*

- What successful CER looks like
- How to translate/disseminate CER information (what works) for doctors and patients

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# Axillary Dissection vs No Axillary Dissection in Women With Invasive Breast Cancer and Sentinel Node Metastasis

## A Randomized Clinical Trial

Armando E. Giuliano, MD; Kelly K. Hunt, MD; Karla V. Ballman, PhD; Peter D. Beitsch, MD; Pat W. Whitworth, MD; Peter W. Blumencranz, MD; A. Marilyn Leitch, MD; Sukamal Saha, MD; Linda M. McCall, MS; Monica Morrow, MD

2011;305(6):569-575.

Annals of Internal Medicine | Medicine and Public Issues

# Comparative Effectiveness Research: A Report From the Institute of Medicine

Harold C. Sox, MD, and Sheldon Greenfield, MD

## Definition of CER

The IOM committee quickly settled on a working definition of CER, which consisted of the elements of earlier definitions reduced to 2 sentences:

> CER is 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.

*Ann Intern Med.* 2009;151:203-205.
This article was published at www.annals.org on 30 June 2009.

- **Comparators: Current practice**
  - **Aids doctors and patients**
  - **Smaller difference**

# Characteristics of Published Comparative Effectiveness Studies of Medications

Michael Hochman, MD
Danny McCormick, MD, MPH

JAMA, March 10, 2010- Vol 303, No 10

# *Previously Excluded Subgroups*

- Women
- Minorities
- Children
- *Patients with multiple co-morbidities*

# *Understudied Sources of Individual Patient Variation*

Heterogeneity of treatment effects can be due to variables that include:

- Biologic causes (e.g. biomarkers, stages detected by imaging, differences in metabolism, etc.).
- Disease severity (i.e. those sicker at baseline respond more favorably)

# *Understudied Sources of Individual Patient Variation (cont')*

- Comorbidity, as a determinant of response due to:
  - Competing risk for mortality or other outcomes
  - Disease-disease interactions
  - Drug-drug interactions
  - Burden of polypharmacy

# *Understudied Sources of Individual Patient Variation (cont')*

- Personal, cultural
- Adherence to treatment

# *Understudied Sources of Individual Patient Variation (cont')*

- Functional status, quality of life, resilience
- Social support
- Depression or other mental health problems
- Medical context, e.g.
  - Willingness and ability to work with providers to optimize/tailor treatments

## Description of Study Measures (n = 1361)

| Study Variables | No. of Items | Range | Mean | SD | Cronbach Alpha |
|---|---|---|---|---|---|
| Total Illness Burden Index (TIBI)*§ | 9 | 0–25 | 5.1 | 3.5 | 0.69 |
| Passivity (PDHCO)*¶ | 13 | 0–100 | 52.9 | 18.0 | 0.75 |
| Functional status (PFI-10)*‖ | 10 | 0–100 | 64.4 | 29.7 | 0.93 |
| Depressive symptomatology (CES-D)* ** | 11 | 0–33 | 12.5 | 8.0 | 0.90 |
| Perceived diabetes burden (Diabetes Burden Scale)*†† | 8 | 0–100 | 39.7 | 28.9 | 0.94 |

Relationship of Composite Potential for Benefit Scale to Adherence to Treatment, Glycemic Control at Baseline (n = 1361)

| Levels of Potential for Benefit Scale* | Adherence to Treatment† Mean (SE) | HbA1c <7%‡ % (SE) | HbA1c§ Mean (SE) |
|---|---|---|---|
| Quartile 1 (highest) | 0.55 (0.03)¶ | 50.2 (2.9)¶ | 7.39 (0.09)¶ |
| Quartile 2 | 0.36 (0.03)‖ | 44.6 (2.8)‖ | 7.57 (0.09)‖ |
| Quartile 3 | 0.24 (0.02)** | 44.1 (2.9)‖ | 7.69 (0.09)‖ |
| Quartile 4 (lowest) | 0.18 (0.03)** | 38.0 (2.9)‖ | 7.75 (0.09)‖ |
| $R^2$ | 0.16 | 0.12 | 0.19 |
| $F_{(3,1358)}$ | 37.89†† | 2.87†† | 2.81‡‡ |

# *Types of CER Studies*

**Trials**

- Classic RCT
- Pragmatic/ Practical
- Adaptive

# *Types of CER Studies*

**Trials**

- Stratified
  (Heterogeneity of Treatment Effects)
- N of One
- Cluster

# *Types of CER Studies*

**Observational Studies**

- From Registries
- From Databases
- Assemble

# *OS Required*

- Good data
- Composites
- Re-design or propensity scores

The NEW ENGLAND JOURNAL of MEDICINE

# Equipoise and the Dilemma of Randomized Clinical Trials

Franklin G. Miller, Ph.D., and Steven Joffe, M.D., M.P.H.

N Engl J Med 2011; 364:476-480 | February 3, 2011

# *CEASAR Study: AIM 1*

To compare the effectiveness of *contemporary* surgical and radiation techniques for localized PCa in terms of the 6- and 12-month patient-reported outcomes, side-effects and complications of treatment.

# *CEASAR Study: AIM 2*

To identify patient level characteristics that may influence comparative effectiveness:

- Race
- Co-morbid conditions
- Socio-economic status
- Personality profile

# *CEASAR Study: AIM 3*

To assess how the comparative effectiveness of the various therapies varies by the quality of care received

WWW.ARCHINTERNMED.COM

# Less Is More

## Severity of Comorbidity and Non-Prostate Cancer Mortality in Men With Early-Stage Prostate Cancer

Daskivich T, Sadetsky N, Kaplan SH, Greenfield S, Litwin M

**Table. Survival Rates and Hazard Ratios for Death for Non–Prostate Cancer Mortality by Global TIBI-CaP Score**

| TIBI-CaP Score | No. of Men | No. of Nonprostate Deaths Within 6 Years | Non–Prostate Cancer Survival Rate at 6 Years, % | Hazard Ratio (95% CI)[a] |
|---|---|---|---|---|
| 0-2 | 1178 | 59 | 94 | 1 [Reference] |
| 3-5 | 1136 | 134 | 86 | 2.19 (1.54-3.11) |
| 6-8 | 429 | 93 | 73 | 3.68 (2.53-5.39) |
| 9-11 | 114 | 34 | 62 | 4.81 (2.93-7.88) |
| ≥12 | 43 | 14 | 59 | 10.29 (5.44-19.46) |

Abbreviations: CI, confidence interval; TIBI-CaP, Total Illness Burden Index for Prostate Cancer.
[a]Hazard ratios were calculated by Cox proportional hazards model controlling for age, education, income, race, and D'Amico tumor risk category.

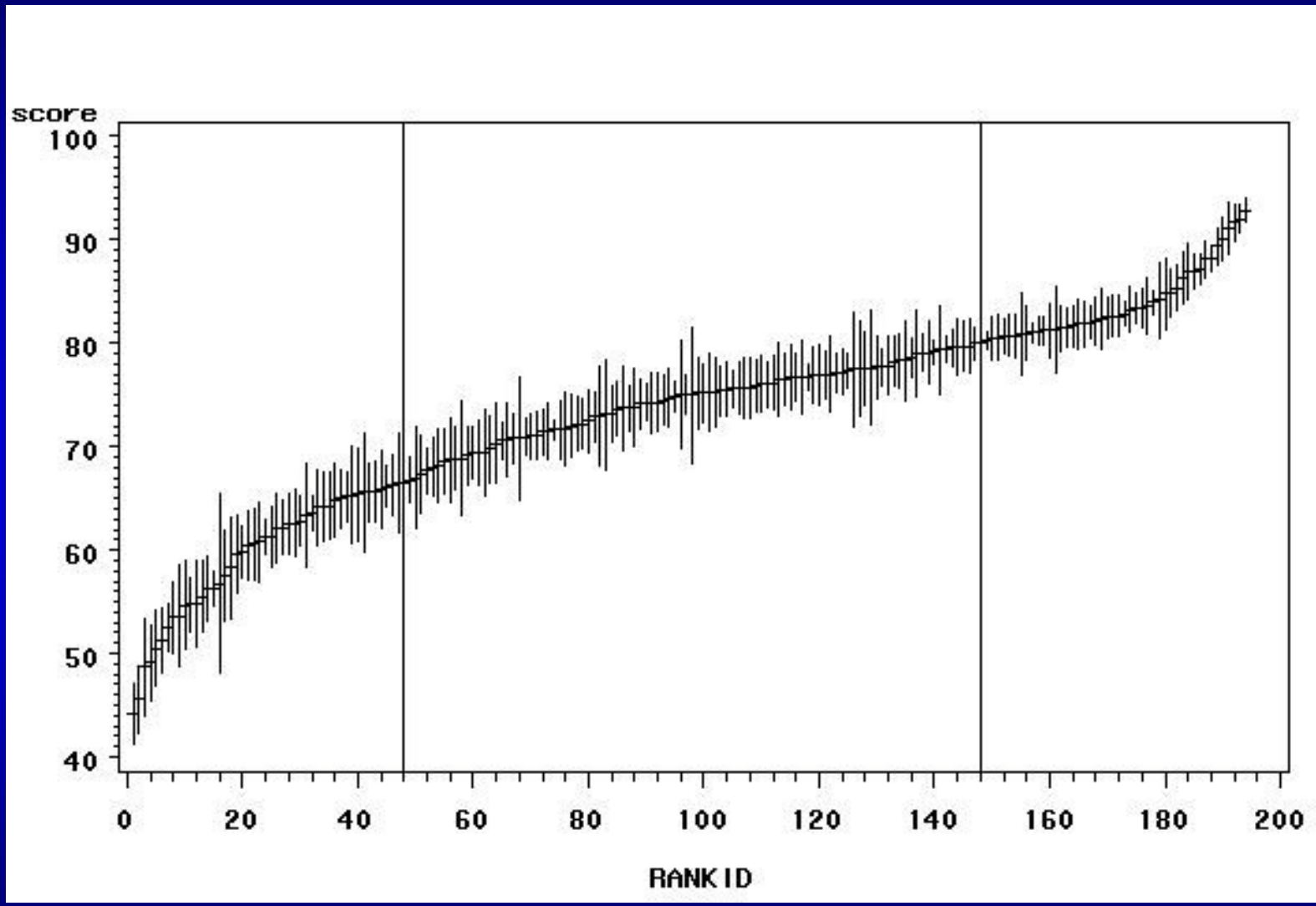
ORIGINAL ARTICLE

# Improving the Reliability of Physician Performance Assessment

## *Identifying the "Physician Effect" on Quality and Creating Composite Measures*

*Sherrie H. Kaplan, PhD, MPH,* John L. Griffith, PhD,† Lori L. Price, MS,†*
*L. Gregory Pawlson, MD, MPH,‡ and Sheldon Greenfield, MD**

*Medical Care* • Volume 47, Number 4, April 2009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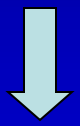
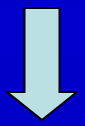
# *Breakdown of Steps from the Lab to the Office*

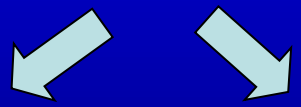
Laboratory



Clinical Observations



Small Trials



Multiple RCTs
(New & Old Forms)

Observational
(New & Old
Forms)

# *Breakdown of Steps from the Lab to the Office*

Data Synthesis

(Systematic Review, Decision Analysis, etc.)

Guidelines

Performance Measures

(Quality)

# *Acceptance*

- Clinical receptivity to new forms of evidence
- Standards for observational studies
- Standards for systematic reviews

# *Acceptance*

- Standards for guidelines
- Separate evidence from valuation
- Consumer and benefit/ risk

**Training patients/doctors/journalists**

***or***

**How to calculate and interpret NNT**

# Number Needed to Treat Approach—Montori @ Mayo

Prepared exclusively for _____

## 1 What goes into figuring out my risk of having a heart attack in the next 10 years?

- Age
- Sex
- Years of diabetes
- Smoking
- Hemoglobin A1C
- Blood pressure
- Cholesterol
- Protein in your urine

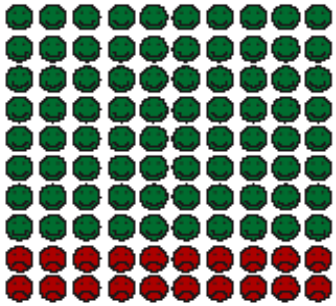
## 2 What is my risk of having a heart attack in the next 10 years?

### NO STATIN

80 people DO NOT have a heart attack (green)

20 people DO have a heart attack (red)

The risk for 100 people like you who DO NOT take statins.



### YES STATIN

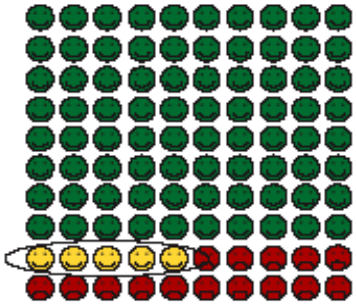
80 people still DO NOT have a heart attack (green)

5 people AVOIDED a heart attack (yellow)

15 people still DO have a heart attack (red)

85 people experienced NO BENEFIT from taking statins

The risk for 100 people like you who DO take statins.



had a heart attack

avoided a heart attack

didn't have a heart attack

## 3 What are the downsides of taking statins (cholesterol pill)?

- Statins need to be *taken every day* for a long time (maybe forever).
- Statins cost money. (to you or your drug plan)
- **Common side effects:** nausea, diarrhea, constipation (most patients can tolerate)
- **Muscle aching/stiffness:** 5 in 100 patients (some need to stop statins because of this)
- **Liver blood test goes up** (no pain, no permanent liver damage): 2 in 100 patients (some need to stop statins because of this)
- **Muscle and kidney damage:** 1 in 20,000 patients (requires patients to stop statins)

## 4 What do you want to do now?

- ☐ Take (or continue to take) statins
- ☐ Not take (or stop taking) statins
- ☐ Prefer to decide at some other time

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# Quality of Care—How Good Is Good Enough?

Harold C. Sox, MD
Sheldon Greenfield, MD

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# *Conclusion*

- Good CER is the first step and first goal
- Translation through CPG and SRS will rise in importance
- All steps can/should include patients/consumers

# *Conclusion*

- Patients/consumers/doctors need training
- The goals can be realized