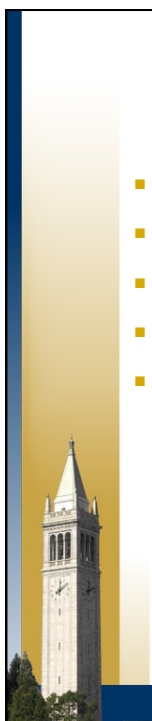




BERKELEY CENTER
FOR HEALTH TECHNOLOGY

**Comparative Effectiveness Research:
Clinical Evidence and Economic Incentives**
University of Paris-Descartes
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OVERVIEW

- The institutional and economic context
- Why comparative effectiveness research (CER)?
- What is CER?
- CER and economic incentives
- Risks and potential for CER in the USA

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The Institutional and Economic Context

Cost Growth in Health Care

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Quality

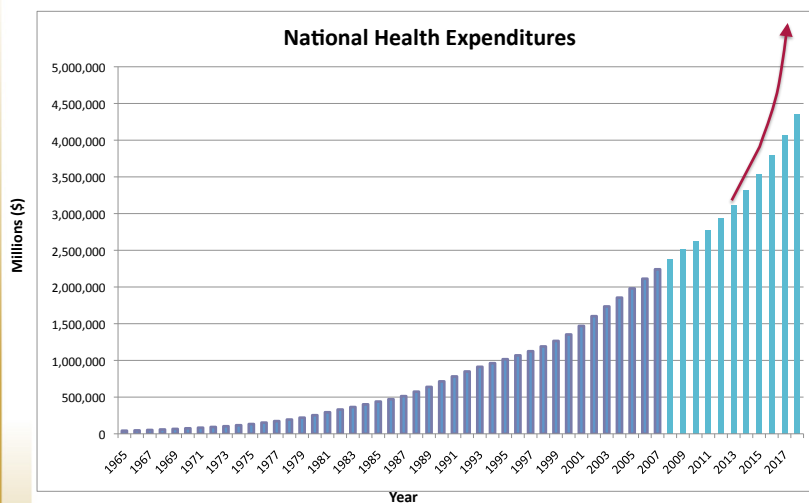
- Does higher cost reflect higher quality and effectiveness?
- Geographic variations in expenditures

Innovation

- The contribution of new technology and clinical uncertainty
- Stronger incentive mechanisms that lack evidence

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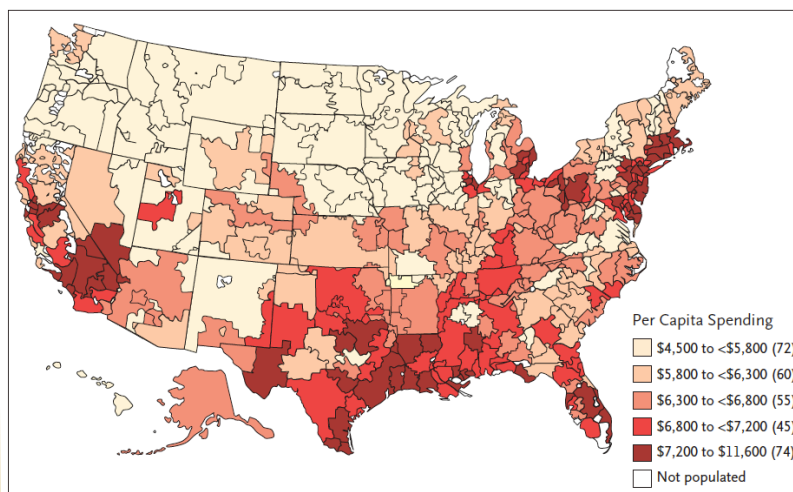
Accelerating Expenditures on Health Care



Source: Centers for Medicare and Medicaid Services, Office of the Actuary, 2009.

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Unjustified Variation in per-Person Expenditures



Source: Dartmouth Atlas of Health Care

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Is CER the solution? Why?

Issues

There is **widespread uncertainty** over what works best:

- Despite huge investment in research, most uses of most therapies have not been studied
- Most drug studies compare effectiveness against placebo, not against major competitors
- Many medical devices have no clinical trial support
- There are few studies of drugs v. other interventions

Source of Blame?

Growing consensus that **new clinical technologies** are major **source of cost growth** (e.g. not profits, waste)

Source for Solution?

Policymakers prefer to **support more research** than to directly **change the economic incentives** that foster rapid development, adoption, and high pricing for therapies

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The Market Context: Incentives without Evidence

Issue

While policymakers prefer *evidence without incentives*, the market for health insurance is strengthening *incentives without evidence*

Source of Blame?

Employers, state governments, and individuals are focused on *cost moderation at any cost*

- Stronger prior authorization barriers
- Higher consumer cost sharing: deductibles, coinsurance
- Efforts to restrain physician, hospital, and drug prices

Source for Solution?

To date, these efforts have **not** focused on *differentially favoring* more effective treatments and *differentially disfavoring* less effective treatments

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What is Comparative Effectiveness Research?

Narrow view

- Head-to-head comparisons of competing drug therapies

Broader view

- Compare drug to non-drug therapies
- Compare alternative treatments for a broad range of conditions, many of which do not involve drugs
- Compare effectiveness of policy interventions (payment methods)

Broadest view

- Clinical effectiveness and cost-effectiveness (CEA)

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Encouraging A Broader Set of Research Designs

Randomized clinical trial

- Gold Standard
- Too long, too expensive for many uses

"Practical clinical trials"

- Shorter trials with intermediate endpoints
- Compare performance against alternative therapies, not placebo
- Look at performance for patient subgroups (by age, gender, severity)

Patient registries & observational studies

- Also use claims data from Medicare and private insurers

Systematic reviews of the existing studies

- Many clinical studies are not published, especially negative studies funded by manufacturers

Dilemma: Is desire for more endpoints, comparisons against more competing therapies, patient subgroups *incompatible* with desire for faster, cheaper studies?

Research Priorities

Compare the effectiveness of...

- ✓ Treatment strategies for atrial fibrillation including surgery, catheter ablation, and pharmacologic treatment
- ✓ Different treatments (...) for hearing loss in children and adults, especially individuals with diverse cultural, language, medical, and developmental backgrounds
- ✓ Primary prevention methods, such as exercise and balance training, versus clinical treatments in preventing falls in older adults of varying risks
- ✓ Upper endoscopy utilization and frequency for patients with gastroesophageal reflux disease on morbidity, quality of life, and diagnosis of esophageal adenocarcinoma
- ✓ Dissemination and translation techniques to facilitate the use of CER by patients, clinicians, payers, and others
- ✓ Comprehensive care coordination programs, such as the medical home, and usual care in managing children and adults with severe chronic disease, especially in populations with health disparities
- ✓ Different strategies of introducing biologics into the treatment algorithm for inflammatory diseases, including Crohn's disease, ulcerative colitis, rheumatoid arthritis and psoriatic arthritis
- ✓ Various screening, prophylaxis, and treatment interventions in eradicating methicillin-resistant *Staphylococcus aureus* (MRSA) in communities, institutions, and hospitals
- ✓ Management strategies for localized prostate cancer (e.g. active surveillance, radical prostatectomy) on survival, recurrence, side effects, quality of life, and costs
- ✓ Pharmacologic and non-pharmacologic treatments in managing behavioral disorders in people with Alzheimer's disease and other dementias in home and institutional settings

Source: Institute of Medicine. "Initial National Priorities for Comparative Effectiveness Research." (2009)

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CER and Economic Incentives: Overview

Common Questions about CER and Incentives

- Why economic incentives?
- Why not FDA as central player in CER?

Current Incentive Mechanisms for Insurers

Coverage policy and "conditional coverage"

Insurance benefit design and consumer cost sharing

Physician payment methods and hospital contracting

Drug pricing and payment methods

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Why Economic Incentives?

How can we promote CER findings?

Without Incentives

Some CER findings will be unequivocal and will be put into practice by physicians without incentives:

- Therapies that have very high risks or zero benefit
- Therapies that have very high effectiveness

✧ *Here the only need is for educational initiatives*

With Incentives

But most CER findings will:

- Be equivocal (e.g., risks v. benefits, effects in subpopulations)
- Interfere with established patient preferences (more is better)
- Interfere with physician practice (paid more to do more)

✧ *Here, unless evidence is combined with incentives, the evidence will only influence practice very slowly, or never*

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Evidence without Incentives: Example

COURAGE Trial under fire by Cardiologists

MARCH 2007

New England Journal of Medicine releases the results from the COURAGE trial:

"...the most common heart surgery (stent implant) yielded **no additional benefits** when patients were treated with a cocktail of generic drugs for chronic chest pain"

APRIL 2007

Stent implant surgeries decline by 13% in the US

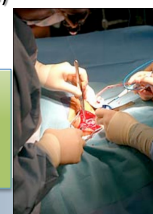
MARCH 2008

Stent implant surgeries return to their pre-COURAGE results levels (250,000 per month)

On average, cardiologists were reimbursed **\$900 per stent procedure**, compared with minimal reimbursement for drug prescription.

It is estimated that **\$5 billion** of \$15 billion spent on stent surgeries is **wasted** due to these financial incentives.

Source: KJ Winstein, "A Simple Health-Care Fix Fizzles Out," *Wall Street Journal*, Feb. 11, 2010.



Why not the Food and Drug Administration (FDA)?

What does it do?

- The FDA is charged with evaluating safety and effectiveness of drugs and devices
- It focuses on RCT and excludes costs

What does it NOT do?

- The FDA does not compare effectiveness against alternative therapies except in limited contexts
- It typically focuses on one indication and does not evaluate or influence off-label use
- FDA is under constant attack for even its limited role in regulating access (by manufacturers, patient advocacy groups, anti-governmental organizations)

CER & the FDA Today

CER debate *does* influence FDA demands for types of study designs (e.g., endpoints, perhaps comparison therapies)

Insurer Incentive Mechanisms: Coverage Policy

Current Coverage Policy

- Insurers deny coverage to therapies they deem ineffective or cosmetic (must be 'medically necessary')
- They do not directly deny coverage due to cost
- They do not directly use threat of denial to influence price

Coverage Policy with CER

- Most CER results will be too equivocal to be incorporated into coverage policy (e.g., to lead to complete coverage denial)
- However, CER results may be embedded into 'conditional coverage policies'
 - ✓ Prior authorization
 - ✓ 'Coverage with evidence development'

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Conditional coverage policy

Prior Authorization

- Coverage is limited to:
 - particular types of patients (by age, diagnosis, severity),
 - types of settings (physician specialty),
 - sequence of treatments
 - **Step therapy:** cheaper alternative must have been found to be ineffective
- **Burden of proof** lies with physician to document need and appropriateness for each patient (though there is appeals mechanism)

Coverage with evidence development (CED)

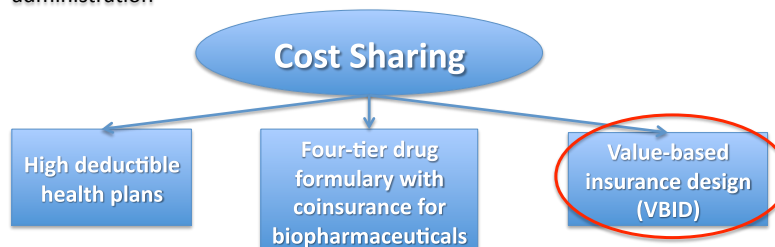
- Some therapies have been given coverage by Medicare contingent on enrolling patients in clinical trial or data registry, with intent of evaluating coverage decision based on data that emerges from these studies
- **Challenge:** who should pay for these trials and registries?

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Benefit Design and Consumer Cost Sharing

There has been major growth in patient responsibility for costs of care in the past decade:

- Employers seek to moderate premium increases
- Ideology of 'consumer driven health care' embraced by Bush administration



Value-Based Insurance Design:

Efforts to remove or reduce cost sharing for most effective drugs and tests

- Chronic illness medications where compliance is a problem
- Preventive tests and interventions

All VBID initiatives to date have focused on therapies where *effectiveness is non-controversial*

Physician Payment and Contracting Methods

Efforts to shift physician payment from volume to 'value'...

Bundled 'episode' payment

- Acute interventions: orthopedic surgery, interventional cardiology
- Acute conditions: maternity, lower back pain
- Chronic conditions: diabetes

Pay-for-Performance

- Bonus based on quality metrics and efficiency (cost) trends
- "Medical home" payments to support primary care practices
- Renewed interest in global capitation payment

These initiatives are only loosely based on evidence of effectiveness, but it is much easier to adjust payment to new CER evidence if the basis of payment already includes quality, appropriateness

Question: *Is there a role for limited contracting ("Center of Excellence") for high cost and high-intensity procedures, based on CER results?*

Drug Pricing and Payment Methods

The US lags behind Europe in linking drug pricing to *evidence of effectiveness*

- Emphasis has been on generic substitution and brand price discounts where there exist therapeutic alternatives (formulary strategy)
- Insurers have almost no leverage with biopharmaceuticals
- Linking coverage to companion diagnostic test (Herceptin)
- European experiments being watched (Velcade)

Payment Reform?

Payment Reform & CER

Principles of 'value based pricing' imply that drug prices should differ according to indication and patient population to the extent CER evidence highlights differences in effectiveness

Evidence

Price

- ✓ Linking evidence to price gives better incentives than linking it to coverage, consumer cost sharing, or physician payments
- ✓ Encourage manufacturers to conduct follow-on studies and develop targeted therapies

Conclusion: Risks of CER

CER is being over-sold

- Studies will be expensive, will take years, and will often produce equivocal results
- There is no reason to assume the results will lead to utilization reductions rather than utilization increases

Illusion

CER suffers from cyclical thinking

- When 'solutions' are over-sold, the public and policymakers become disillusioned with their failure to achieve unrealistic expectations, and may shift quickly to the next 'solution'

Disillusion

CER must be a long term investment, not short term cost control mechanism

Conclusion: Potential for CER

Whatever be the direct impact of CER on use and cost of particular therapies, it can broaden the policy dialogue in important ways:

- Highlight the variation in performance across therapies and across patient subgroups
- Highlight importance of setting priorities and making tradeoffs
- Dispel the illusion of 'medical necessity' as driving utilization

A Foundation based on CER

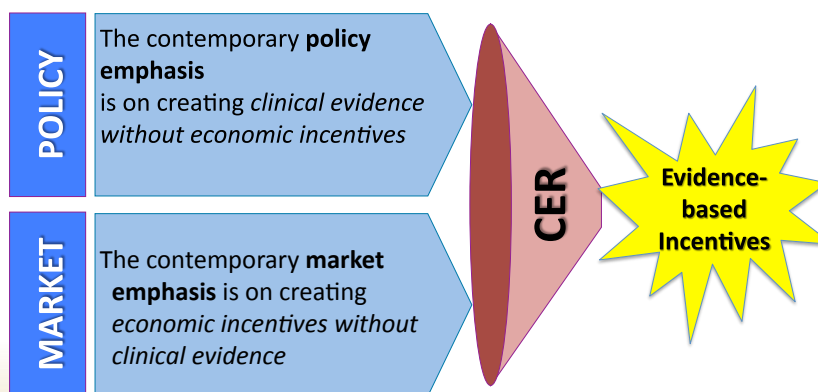
The institutional foundations for ongoing applied clinical research:

- Comparative endpoints rather than placebo trials
- Validation of multiple study designs
- Combine cost assessment with quality assessment

The cultural foundations for continuing research that directly affects coverage, cost sharing, payment, and pricing

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Conclusion: Evidence and Incentives



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