DOES COMPARATIVE-EFFECTIVENESS RESEARCH HAVE LESSONS TO LEARN FROM HEALTH TECHNOLOGY ASSESSMENT?

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Community Health and Epidemiology
Dalhousie University
RETURN ON HEALTH INVESTMENT

Premium Price, Poor Performance

The United States spends more on health care per capita—by far—than any of the other OECD countries. Yet it ranks in the bottom 25% of those countries on life expectancy. by Jeff Levin-Scherz
RETURN ON HEALTH INVESTMENT

Figure 2. Health status and health care spending; persisting differences in level across OECD countries, 2003

Life expectancy at birth (years)

Total health spending, US $ PPP per capita

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HEALTH EXPENDITURES

16% OF GDP IN US
VERSUS
8% - 10% OF GDP IN OTHER INDUSTRIALIZED NATIONS

US HEALTH EXPENDITURES ($US)

<table>
<thead>
<tr>
<th>Year</th>
<th>Per Person</th>
<th>Overall</th>
</tr>
</thead>
<tbody>
<tr>
<td>1980</td>
<td>$1,106</td>
<td>$255 BILLION</td>
</tr>
<tr>
<td>2007</td>
<td>$7,421</td>
<td>$2200 BILLION</td>
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</table>
QUALITY OF CARE VS STATE SPENDING

CONCERN ABOUT RAPID GROWTH:
CONGRESSIONAL BUDGET OFFICE: PREDICTS THAT WITHOUT CHANGES, GOVERNMENT-PAID INSURANCE FOR ELDERLY/POOR:
4% OF GDP IN 2004 ➔ 12% IN 2050

HEALTH EXPENDITURES

NEW HEALTH TECHNOLOGIES – INVESTMENT IN R&D
– US$100 BILLION PER Y IN US &
$100 BILLION EX-US
**WHAT IS COMPARATIVE EFFECTIVENESS RESEARCH?**

$1.1 BILLION- AMERICAN RECOVERY & REINVESTMENT ACT

500 MILLION PER YEAR ANNUALLY

SOLUTION TO CHALLENGES INCLUDING:
- IMPROVING CLINICAL DECISION MAKING
- ENHANCING VALUE FOR MONEY
- CONTAINING COSTS
- REDUCING HEALTH DISPARITIES

US FEDERAL COORDINATING COUNCIL ON CER:
“INFORMATION ON THE RELATIVE STRENGTHS AND WEAKNESS OF VARIOUS MEDICAL INTERVENTIONS. SUCH RESEARCH WILL GIVE CLINICIANS AND PATIENTS VALID INFORMATION TO MAKE DECISIONS THAT WILL IMPROVE THE
COMPARATIVE EFFECTIVENESS RESEARCH

ADDRESS THE FOLLOWING QUESTIONS:

WHAT IS THE CONTEXT FOR CER?

WHAT IS IT?

HOW CAN THE INTERNATIONAL EXPERIENCE IN HTA INFORM THE DEBATE IN THE US?

CAN CER MEET THE OBJECTIVES AND EXPECTATIONS?

SUGGESTIONS FOR A WAY FORWARD (HUMBLY OFFERED)
Drummond et al. IJTAHC 2008 24(3): 244
<table>
<thead>
<tr>
<th>PURPOSE</th>
</tr>
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<tbody>
<tr>
<td>LESSONS FOR THE US FROM INTERNATIONAL EXPERIENCES IN COMPARATIVE EFFECTIVENESS RESEARCH</td>
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</table>

| SPECIFIC OBJECTIVE: |
| TO CRITICALLY COMPARE PROCESSES OF COMPARATIVE EFFECTIVENESS RESEARCH IN FIVE JURISDICTIONS |

| FOCUS |
| PROCESSES TO ASSESS VALUE OF NEW MEDICATIONS |
| OTHER AREAS OF COMPARATIVE EFFECTIVENESS |
METHODS

COMPREHENSIVE LITERATURE REVIEW

• IN EACH JURISDICTION:
  • CENTRALIZED REVIEW OF MEDICATIONS
  • OTHER ELEMENTS OF COMPARATIVE EFFECTIVENESS RESEARCH

FOCUS: GOVERNMENT AGENCIES DOING HTA

KEY INFORMANT INTERVIEWS
JURISDICTIONS CONSIDERED

35 million

22 million

15 million

9 million

6 million
EXPENDITURES ON HEALTH AND DRUGS

![Chart showing expenditures on health and drugs as a percentage of GDP and as a percentage of health expenditure on drugs for Canada, Australia, Sweden, the Netherlands, and the US.]

- Canada
- Australia
- Sweden
- Netherlands
- US
# CENTRALIZED REVIEW OF NEW MEDICATIONS

<table>
<thead>
<tr>
<th>COUNTRY - ACRONYM</th>
<th>MANDATE</th>
</tr>
</thead>
</table>
| CANADA – CEDAC    | - IMPARTIAL ADVICE ON EFFICACY, COST-EFFECTIVENESS  
|                   | - REDUCE DUPLICATION IN THE REVIEW PROCESS |
| AUSTRALIA – PBAC  | - IMPARTIAL ADVICE ON EFFICACY, COST-EFFECTIVENESS  
|                   | - EQUITY |
| SWEDEN – TLV      | - DETERMINES WHETHER A DRUG WILL BE REIMBURSED  
|                   | - EQUITY |
| NETHERLANDS – CFH | - COST-EFFECTIVENESS, PRICING  
|                   | - EQUITY |
| SCOTLAND – SMC    | - COST-EFFECTIVENESS  
|                   | - EQUITY |
CONSTITUTION AND GOVERNANCE

ADVISORY:

CANADA (CEDAC): ADVISES PROVINCIAL FORMULARIES
AUSTRALIA (PBAC): ADVISES MINISTER OF HEALTH
NETHERLANDS (CFH): ADVISES MINISTER OF HEALTH

REGULATORY:

SWEDEN (TLV)
SCOTLAND (SMC)*

* NEGATIVE DECISIONS ARE ADVISORY
OVERVIEW OF PROCESSES

REVIEW ALL NEWLY LICENSED MEDICATIONS SUBMISSIONS BY MANUFACTURER
- PUBLISHED GUIDELINES
- ALL USE COST PER QALY FRAMEWORK

REVIEW BY IN-HOUSE OR EXTERNAL (OZ) EXPERTS

ASSESSED BY ADVISORY COMMITTEE

RECOMMENDATION

APPEAL PROCESSES
# Committee Composition

<table>
<thead>
<tr>
<th>Country</th>
<th>MD</th>
<th>LAY</th>
<th>Pharmacists</th>
<th>Health Economics</th>
<th>Pharmacy</th>
<th>Health Administrators / Ethics</th>
<th>Public Health / Epidemiology</th>
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<tr>
<td><strong>Australia</strong></td>
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<td>1</td>
<td>2</td>
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<tr>
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<td></td>
<td><strong>Not reported</strong></td>
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<tr>
<td><strong>Scotland</strong></td>
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<td>6</td>
<td>3</td>
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<tr>
<td><strong>Sweden</strong></td>
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EVALUATION PROCESSES

CRITERIA ASSESSED

STAGE 1
EFFICACY
SAFETY
CLINICAL NEED/AVAILABILITY OF ALTERNATIVE TREATMENTS

STAGE 2
COST-EFFECTIVENESS - IMPLICIT THRESHOLDS FOR QALY
BUDGET IMPACT

NONE SPECIFY RELATIVE IMPORTANCE OF CRITERIA
USE OF EVIDENCE

ALL PRIORITIZE EFFICACY DATA FROM RANDOMIZED TRIALS

OBSERVATIONAL DATA CONSIDERED AS SUPPLEMENTARY

INDIRECT COMPARISONS ACCEPTABLE IN THE ABSENCE OF HEAD-TO-HEAD EVIDENCE
### SUMMARY OF STRUCTURES

<table>
<thead>
<tr>
<th>Country</th>
<th>Stated Purpose – IE More Than Technical</th>
<th>Authority</th>
<th>Centralization</th>
<th>Composition % MDS</th>
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<tbody>
<tr>
<td>Canada</td>
<td>Reduce Duplication</td>
<td>Advisory</td>
<td>Decentralized</td>
<td>70</td>
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<tr>
<td>Australia</td>
<td>Equity</td>
<td>Advisory</td>
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<td>Expedite Process</td>
<td>Regulatory</td>
<td>Somewhat Decentralized</td>
<td>40</td>
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</table>

None Have Delisting Process
DAYS TO REGULATORY APPROVAL & TO REIMBURSEMENT

- Canada (HC)
- Australia (TGA)
- EMEA (SWE; NTHLDS; SCOT)
- US (CDER)

Chart showing days to regulatory approval and reimbursement for different countries.
## COMPARATIVE EFFECTIVENESS RESEARCH

<table>
<thead>
<tr>
<th>countries</th>
<th>Medications</th>
<th>Other Health Technologies</th>
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<tbody>
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<tr>
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<td>X</td>
<td></td>
</tr>
<tr>
<td>SWEDEN</td>
<td>X</td>
<td>X</td>
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<tr>
<td>NETHERLANDS</td>
<td>X</td>
<td></td>
</tr>
<tr>
<td>SCOTLAND</td>
<td>X</td>
<td></td>
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</table>

<table>
<thead>
<tr>
<th>HTA – Reimbursement Review</th>
<th>Promoting Safe and Effective Use</th>
<th>Multi-Technology</th>
<th>Pragmatic Trials (Conditionally Funded Field)</th>
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<tbody>
<tr>
<td>CANADA</td>
<td>X</td>
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<td>X</td>
</tr>
<tr>
<td>AUSTRALIA</td>
<td>X</td>
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<td>X</td>
</tr>
<tr>
<td>SWEDEN</td>
<td>X</td>
<td>X</td>
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</tr>
<tr>
<td>NETHERLANDS</td>
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<tr>
<td>SCOTLAND</td>
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</tr>
</tbody>
</table>

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SUMMARY COMPARISON OF HTA FOR MEDICATIONS

COMMONALITIES – GREATER THAN THEIR DIFFERENCES

- ALL USE COST PER QALY
- SIMILAR CRITERIA, NONE EXPLICIT ABOUT WEIGHTING
- NONE USE EXPLICIT ICER THRESHOLD...BUT, THERE IS IMPLICIT VALUE
- POSITIVE LISTING RECOMMENDATION; NO DELISTING PROCESS
- LINK TO PRICING

DIFFERENCES
- TRANSPARENCY OF DECISIONS

- COMMITTEE MAKE-UP
SUMMARY COMPARISON OF HTA FOR MEDICATIONS

COMPARISONS OF FORMULARIES

DIFFICULT TO MAKE FAIR COMPARISONS

IMPACT ON TREATMENT AND PATIENT OUTCOMES

COMPLETELY UNKNOWN
INSIGHTS

GATEKEEPER ROLE MUCH STRONGER THAN DISINVESTMENT (DELISTING THERAPIES MUCH MORE DIFFICULT)

WITHOUT A LINK TO REIMBURSEMENT, HTA PROCESSES HAVE SELECTIVE EFFECTS ON PRACTICE

USING HTA TO CONTAIN COSTS DOES NOT WORK

CONSISTENCY OF EFFORT IN TRANSLATION ACTIVITIES AND RECEPTOR CAPACITY
HHS SECRETARY KATHLEEN SEBELIUS: CER WOULD NOT LEAD TO CENTRALIZED COVERAGE DECISIONS, CONSISTENT WITH 2003 MEDICARE MODERNIZATION ACT.

SHE EMPHASIZED THE GOAL OF ANY GOVERNMENT-LED CER ENTERPRISE WOULD BE TO: “PROVIDE ADDITIONAL INFORMATION TO PATIENTS AND PROVIDERS TO HELP THEM MAKE THE BEST DECISIONS POSSIBLE REGARDING TREATMENT OPTIONS.”
MY OPINION ON IMPROVING CLINICAL DECISION MAKING

HHS SEBELIUS’S POSITION DOES LITTLE TO STRENGTHEN THE LINK BETWEEN RESEARCH FINDINGS AND CHANGING MEDICAL PRACTICE.

WITHOUT EXPLICITLY TYING RESULTS OF CER TO THE USE AND AVAILABILITY OF HEALTH TECHNOLOGIES: CER ENTERPRISE LIMITED TO INFORMATIONAL ROLE
CONTAINING COSTS

CER - AN INDIRECT METHOD OF CONTAINING COSTS
- MAY HAVE THE OPPOSITE IMPACT BY LEADING TO GREATER USE OF SERVICES DEEMED CLINICALLY BENEFICIAL.
- INDIRECT APPROACH IS UNLIKELY TO BE SUCCESSFUL IF COST-CONTAINMENT IS A GOAL:
  - POLICY-MAKERS AND LEGISLATORS NEED TO ADDRESS HEAD-ON
7 SHARED CHARACTERISTICS OF HTA THAT MAY APPLICABLE TO CER IN THE US (LEVY ET AL, PHARMACOECONOMICS, IN PRESS)

1. PROCESS MUST BE RESPONSIVE TO STAKEHOLDERS’ INTERESTS:
   - TURN-AROUND TIME FOR ASSESSMENTS MUST BE MINIMIZED
   - TRANSPARENCY MUST BE MAXIMIZED
   - PROCESS MUST BE CONSIDERED FAIR USING UNIVERSALLY AGREED STANDARDS
   - PROCESS MUST BE MODIFIABLE BASED ON STAKEHOLDERS’ REQUIREMENTS

2. THE ASSESSMENT OF PHARMACEUTICALS AND OTHER MEDICAL TECHNOLOGIES PRESENTS DIFFERENT CHALLENGES A
   - MAY NEED TO BE MANAGED SEPARATELY

3. The HTA process following regulatory approval can delay market access to new technologies,
   ➔ Closer integration between regulatory approval and HTA processes is being explored internationally.

4. There is a direct or indirect link to reimbursement in the jurisdictions explored.
   ➔ Without this link the role of CER in the US will remain advisory.

5. Most jurisdictions benefit from a single payer that is informed by the process –
   Given the diverse multipayer environment, US CER may focus on generating effectiveness evidence and allowing each payer to use its own costing.
7 SHARED CHARACTERISTICS OF HTA THAT MAY APPLICABLE TO CER IN THE US (LEVY ET AL, PHARMACOECONOMICS, IN PRESS)

6. A COMMON METRIC FOR ASSESSING INTENDED AND UNINTENDED EFFECTS OF TREATMENT ALLOWS COMPARISON ACROSS DIFFERENT TECHNOLOGIES. E.G., THE QALY

7. THE STATED FOCUS OF CER ON THERAPEUTIC BENEFIT AMONG ‘HIGH-PRIORITY POPULATIONS’ WILL BE DIFFICULT TO ACHIEVE BECAUSE EPIDEMIOLOGICAL EVIDENCE OF DIFFERENCES IN THERAPEUTIC BENEFIT AMONG SUBGROUPS IS DETECTED THROUGH EFFECT MODIFICATION, OR MORE SPECIFICALLY, STATISTICAL EVIDENCE OF EFFECT MEASURE
EXPECTATIONS

- “THIS UNIQUE OPPORTUNITY TO INVEST IN A MAJOR COMPONENT OF THE SCIENTIFIC INFRASTRUCTURE FOR IMPROVING HEALTH CARE DELIVERY WILL BE INDISPENSABLE FOR ACHIEVING A HEALTH CARE SYSTEM THAT DELIVERS AFFORDABLE, HIGH-QUALITY CARE FOR ALL AMERICANS.”

- COUNCIL’S EXECUTIVE DIRECTOR AND THE DIRECTOR OF AHRQ (NEJM JUNE 30, 2009)
• THOSE EXPECTATIONS ARE UNLIKELY TO FULFILL CURRENT EXPECTATIONS BECAUSE OF
  - POLITICAL CONSTRAINTS
  - MULTIPLICITY OF OBJECTIVES
  - THE LACK OF COHERENCE BETWEEN THE PROBLEMS FACING THE US HEALTH CARE SYSTEM AND THE SOLUTIONS THAT CER MIGHT INFORM

• AS CURRENTLY CONSTRUED, CER MAY TOUCH ON MANY PROBLEMS FACING US HEALTH CARE → RUNS THE RISK OF SOLVING NONE
WHAT IS TO BE DONE? (HUMBLY OFFERED)

• THE EXTENT TO WHICH CER MEETS EXPECTATIONS DEPENDS CRITICALLY ON THE GOVERNANCE, STRUCTURES AND PROCESSES PUT IN PLACE.

AT LEAST TWO ISSUES NEED TO BE CLARIFIED:

- ETHICAL LENS
- OBJECTIVES
THANK YOU FOR YOUR ATTENTION