Toward a Broader Value Framework for Health Technology Assessment:
Expanding Cost-Effectiveness Analysis

SUSTAINABLE HEALTHCARE: Symposium in Health Economics
ELTE, Faculty of Social Sciences

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Agenda

• Economic definition of value
• Emerging value frameworks
• ISPOR Initiative on US Value Frameworks
Underlying Assumption

• If we want innovators to create valuable new medical technologies, we need to signal them (1) about what we value and (2) that we will reward them in proportion to the value they create.

• Implication: we need to define what we value and how it is measured.
What is “Value”? 

• From an economic perspective:
  – Value is what someone is (actually) willing to pay or forgo to obtain something (opportunity cost)

• Implications:
  – Varies across individuals and over time.
  – Difficult to measure in health care
  – In principle, we need to take an patient incremental insurance perspective
Defining Economic Value for HTA: Standard Definition

What is “economic value”? 

- “Value”= what fully informed patients would be willing to pay (WTP) for a new medicine based on:
  
  1) any cost savings,

  2) life years gained (LYs),

  3) improvements in quality of life or morbidity

  \((2+3) \Rightarrow Quality-adjusted life years--QALYs\)
Defining Economic Value: Broadening the Measure

• What is “economic value”?
• “Value” = what fully informed patients would be willing to pay (WTP)—usually via insurance—for a new medicine based on:
  1) any cost savings,
  2) life years gained (LYs),
  3) improvements in quality of life or morbidity (2+3→QALYs)
  4) productivity gains
  5) reduction in uncertainty due to better data or the value of knowing (e.g., via personalized medicine)
  6) improvements in population-level adherence and uptake (via personalized medicine)
  7) innovation—scientific spillovers
  8) option value--survival creates an option to benefit from future advances;
  9) “value of hope”—paying more for cures
Insurance Perspective (Garber & Phelps, 1997)

• “Implicit in our discussion is the assumption that CE analysis is used to improve decision making at an individual level.
• Ordinarily an apparatus like CEA analysis is unnecessary for individual consumption decisions, in the absence of externalities or public considerations.
• In health care, however, the familiar informational failures are sufficient reason for CE analysis to be performed as an aid to individual decisions.
• A more common application, however, is for decisions about the scope of health insurance: the technique can be used to help determine which forms of health care should be reimbursed by a private or governmental insurer, or provided by a health maintenance organization.
• The optimal CE criterion is equivalent to determining optimal coverage for an actuarially fair insurance policy, under perfect information.”
Second-Panel Volume: Just Released—October 2016
Second Panel on CEA: Impact Inventory

<table>
<thead>
<tr>
<th>Sector</th>
<th>Type of Impact</th>
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<tbody>
<tr>
<td>Formal Health Care Sector</td>
<td>Health outcomes (effects)</td>
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<tr>
<td></td>
<td>Longevity effects</td>
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<td></td>
<td>Health-related quality-of-life effects</td>
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<td></td>
<td>Other health effects (e.g., adverse events and secondary transmission of infections)</td>
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<tr>
<td>Medical costs</td>
<td>Paid for by third-party payers</td>
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<td></td>
<td>Paid for by patients out-of-pocket</td>
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<td></td>
<td>Future related medical costs (patients and providers)</td>
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<tr>
<td></td>
<td>Future unrelated medical costs (patients and providers)</td>
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<td>Informal Health Care Sector</td>
<td>Patient-time costs</td>
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<td></td>
<td>Unpaid caregiver-time costs</td>
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<td></td>
<td>Transportation costs</td>
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<tr>
<td>Non-Health Care Sectors</td>
<td>Productivity</td>
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<tr>
<td></td>
<td>Labor market earnings lost</td>
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<td></td>
<td>Cost of unpaid lost productivity due to illness</td>
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<td></td>
<td>Cost of uncompensated household production</td>
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<tr>
<td>Consumption</td>
<td>Future consumption unrelated to health</td>
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<tr>
<td>Social Services</td>
<td>Cost of social services as part of intervention</td>
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<td>Legal or Criminal Justice</td>
<td>Number of crimes related to intervention</td>
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<tr>
<td></td>
<td>Cost of crimes related to intervention</td>
</tr>
<tr>
<td>Education</td>
<td>Impact of intervention on educational achievement of population</td>
</tr>
<tr>
<td>Housing</td>
<td>Cost of intervention on home improvements (e.g., removing lead paint)</td>
</tr>
<tr>
<td>Environment</td>
<td>Production of toxic waste pollution by intervention</td>
</tr>
<tr>
<td>Other (specify)</td>
<td>Other impacts</td>
</tr>
</tbody>
</table>

Notes on Sources of Evidence:

- □ Included in this Reference Case Analysis From... Perspective?
- □ Sources of Evidence
Agenda

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• ISPOR Initiative on US Value Frameworks
US Drug Value Frameworks

Source: P. Neumann, May 25, 2016
Escalating drug prices have alarmed physicians and the American public and led to calls for government price controls. Less visibly, they have also spawned a flurry of private-sector initiatives designed and other interventions is a positive step. Anger over rising drug prices may be understandable, but it has led some observers to call for setting prices to reflect overall development costs.

Source: Neumann and Cohen, NEJM, 2015
Frameworks use different attributes of value

<table>
<thead>
<tr>
<th>Context/Perspective</th>
<th>Clinical Treatment Guidelines</th>
<th>Shared Decision-Making</th>
<th>Coverage &amp; Payment</th>
<th>Shared Decision-Making &amp; Pricing</th>
<th>Shared Decision-Making</th>
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<tr>
<td>ACA/AHA</td>
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<td>ICER</td>
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<tr>
<td>Sloan Kettering</td>
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<tr>
<td>NCCN</td>
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<td></td>
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</table>

Source: Adapted from P. Neumann, May 25, 2016
Budget impact: ICER--[“Affordability”]

- GDP growth + 1% arbitrary
- All drugs held to same budget cap
- Drugs treating many patients penalized

Source: P. Neumann, May 25, 2016
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In the last few years, a number of value assessment frameworks have been developed as the health care system has moved toward a value-driven approach that focuses on evaluating therapeutic options based on health outcomes, value to the patient, and effectiveness compared with other potential treatment options.

The currently available frameworks, however, are widely diverse in their approaches, and this inconsistency can lead to variable evaluations of treatments.

A need therefore exists for a robust discussion of relevant perspectives and appropriate approaches that (a) are transparent and methodologically sound and (b) involve the input of key stakeholders to guide the development of value assessment frameworks for health care decision making.
Initiative Aims

1. Identify and discuss key methodological and process issues in defining and applying value frameworks to health care resource allocation issues, and

2. Convene a Special Task Force (STF) to collaborate on a white paper that reviews relevant perspectives and appropriate approaches and methods to support the definition and use of high-quality value frameworks.

3. Engage key stakeholders throughout the development of the white paper to help to frame the scope of work for this methodologically-oriented white paper and to review and comment on the STF’s work progress and products.
Initiative on US Value Assessment Frameworks

Steering Committee

Key methodological issues in defining and applying value frameworks to health care resource allocation

Expert Advisory Board

Public Call for Papers

Stakeholder Advisory Panel

Stakeholder Conference (Fall 2016)

Special Task Force

Themed Issue of Value in Health (early 2017)

White paper and commentaries for publication (2017)

Sessions at ASHEcon (Summer 2016)

Rollout at ISPOR Boston (May 2017)
Expert Advisory Board survey question:
Do you believe that cost-utility analysis is a valid approach for measuring the value of healthcare interventions?

83% responded “yes” but most noted qualifications:
1. CUA contains limitations or is incomplete (n=5)
2. CUA is one of many possible approaches (n=3)
3. The approach must utilize appropriate measures (n=3)

Key suggested alternatives to CUA included:
- Expanded/extended/enhanced/modified CUA analysis (n=4)
- Value assessment focused on willingness to pay (WTP) (n=2)
- Alternatives to CUA (e.g. multi-criteria decision analysis) (n=2)
The Special Task Force (STF) will produce a scientific policy white paper that reviews relevant perspectives and appropriate approaches and methods to support the construction and use of high-quality health care value frameworks that will enable more efficient health sector decision-making in the US.
EAB and SAP Survey Question:
Which of the following decision-making contexts are the most important for the STF to consider?

Average score (0= least important, 5= most important)

- Payer level (adaptable to the various insurance sectors in the US)
- Societal level (health sector vs. other)
- Patient-physician shared decision making
- Clinical guidelines (physician as agent for broader clinical/societal considerations)
EAB & SAP Survey Question:
Which of the following potential elements of value are the most important for the STF to consider? (part 1)

Average score (0= least important, 5= most important)

- Budget constraints and affordability concerns
- Disinvestment in inefficient technologies
- Excess burden of raising funds via taxation
- Scientific spillovers
- Risk of contagion
- Value of reduction in uncertainty due to dx accuracy
- Value of hope due to the potential for major treatment benefit

SAP responses  EAB responses
EAB & SAP Survey Question:
Which of the following potential elements of value are the most important for the STF to consider? (part 2)

Average score (0= least important, 5= most important)

- Value of peace of mind due to insurance coverage
- Real option value
- Productivity
- Severity of disease
- Near-term mortality probability

Adherence-improving factors

Direct input from patients

SAP responses
EAB responses
The Value of Knowing and Knowing the Value: Improving the Health Technology Assessment of Complementary Diagnostics

WHITE PAPER
May 2016

Notes:
- Light grey circle: traditional elements of value as considered by HTA
- Dark grey circle: expanded value framework: elements not traditionally considered/measured
- Green line: value from health system perspective
- Red line: value also included in societal perspective

Source: Garrison, L., Mestre-Ferrandiz, J. and Zamora, B., OHE and EPEMED, Forthcoming, June, 2016
Importance of Context

Need to consider each context separately as well as the perspective:

1. Regulatory benefit-risk
2. HTA for coverage and decisions
3. Pricing and reimbursement
4. Clinical treatment guidelines
5. Physician-patient shared decision-making
Three Key Questions for Value Frameworks

Value frameworks should address three key questions:
1. What are the elements of value?
2. How are they measured, evidenced, and valued?
3. How are they aggregated and judged to reach a decision on value?
## Elements of ‘Value’ internationally

<table>
<thead>
<tr>
<th></th>
<th>E&amp;W</th>
<th>Australia</th>
<th>Canada</th>
<th>France</th>
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<tbody>
<tr>
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<td>✓</td>
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<tr>
<td>Alternatives available / unmet need</td>
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<td></td>
<td>✓</td>
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<tr>
<td>Disease severity</td>
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<td>EoL</td>
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<td>✓</td>
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<td>New mode of action</td>
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<td></td>
<td>✓</td>
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<td></td>
<td>✓</td>
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<tr>
<td>Cost savings beyond health care</td>
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<td></td>
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<td>✓</td>
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<tr>
<td>Productivity</td>
<td></td>
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<td></td>
<td>✓</td>
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</table>
How measured, evidenced, and valued/rated?

• Measured:
  • e.g. health effect: Use of QALYs, clinical outcomes, PROs, disease specific

• Evidenced:
  • e.g. health effect: Use of RCTs, observational studies, patient testimony, clinical opinion

• Valued/rated
  • e.g. use of population or patient values
  • e.g. use of categories or discrete scales
Challenges and Next Steps

• Expanding beyond CUA
  – Identifying all relevant elements

• How and how much to monetize—or not?
  – Utilize multi-criteria decision-making (MCDA) or mixed model (quantitative and qualitative)?

• Decision-making
  – Rules (e.g., threshold) vs. type of deliberative process?
Thank you!

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