

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

SUSTAINABLE HEALTHCARE: Symposium in Health Economics
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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) → Quality-adjusted life years--QALYs



Defining Economic Value: Broadening the Measure

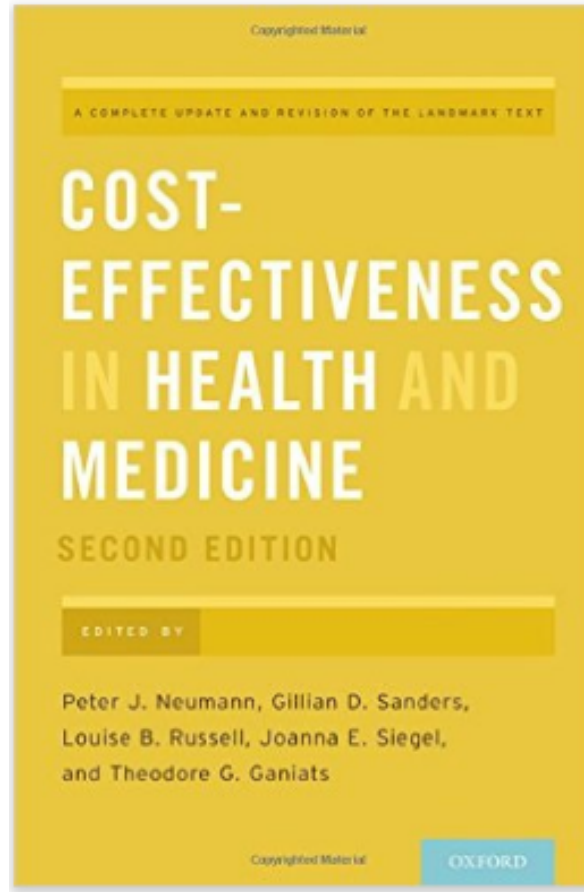
- What is “economic value”?
- “Value”= what fully informed patients would be willing to pay (WTP)—**usually via insurance**—for a new medicine based on:
 - 1) any cost savings,
 - 2) life years gained (LYs),
 - 3) improvements in quality of life or morbidity (2+3→QALYs)
 - 4) productivity gains
 - 5) 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

Figure 1. Impact Inventory Template

Sector	Type of Impact (list category within each sector with unit of measure if relevant) ^a	Included in This Reference Case Analysis From...Perspective?		Notes on Sources of Evidence
		Health Care Sector	Societal	
Formal Health Care Sector				
Health	Health outcomes (effects)			
	Longevity effects	<input type="checkbox"/>	<input type="checkbox"/>	
	Health-related quality-of-life effects	<input type="checkbox"/>	<input type="checkbox"/>	
	Other health effects (eg, adverse events and secondary transmissions of infections)	<input type="checkbox"/>	<input type="checkbox"/>	
	Medical costs			
	Paid for by third-party payers	<input type="checkbox"/>	<input type="checkbox"/>	
	Paid for by patients out-of-pocket	<input type="checkbox"/>	<input type="checkbox"/>	
Health	Future related medical costs (payers and patients)	<input type="checkbox"/>	<input type="checkbox"/>	
	Future unrelated medical costs (payers and patients)	<input type="checkbox"/>	<input type="checkbox"/>	
Informal Health Care Sector				
Health	Patient-time costs	NA	<input type="checkbox"/>	
	Unpaid caregiver-time costs	NA	<input type="checkbox"/>	
	Transportation costs	NA	<input type="checkbox"/>	
Non-Health Care Sectors (with examples of possible items)				
Productivity	Labor market earnings lost	NA	<input type="checkbox"/>	
	Cost of unpaid lost productivity due to illness	NA	<input type="checkbox"/>	
	Cost of uncompensated household production ^b	NA	<input type="checkbox"/>	
Consumption	Future consumption unrelated to health	NA	<input type="checkbox"/>	
Social Services	Cost of social services as part of intervention	NA	<input type="checkbox"/>	
Legal or Criminal Justice	Number of crimes related to intervention	NA	<input type="checkbox"/>	
	Cost of crimes related to intervention	NA	<input type="checkbox"/>	
Education	Impact of intervention on educational achievement of population	NA	<input type="checkbox"/>	
Housing	Cost of intervention on home improvements (eg, removing lead paint)	NA	<input type="checkbox"/>	
Environment	Production of toxic waste pollution by intervention	NA	<input type="checkbox"/>	
Other (specify)	Other impacts	NA	<input type="checkbox"/>	



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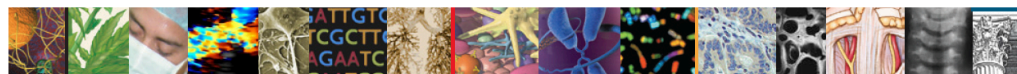


US Drug Value Frameworks



Source: P. Neumann, May 25, 2016





The NEW ENGLAND JOURNAL of MEDICINE

Perspective

Measuring the Value of Prescription Drugs

Peter J. Neumann, Sc.D., and Joshua T. Cohen, Ph.D.

Escalating drug prices have alarmed physicians and the American public^{1,2} 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 research, development, and pro-

Source: Neumann and Cohen, NEJM, 2015

Frameworks use different attributes of value



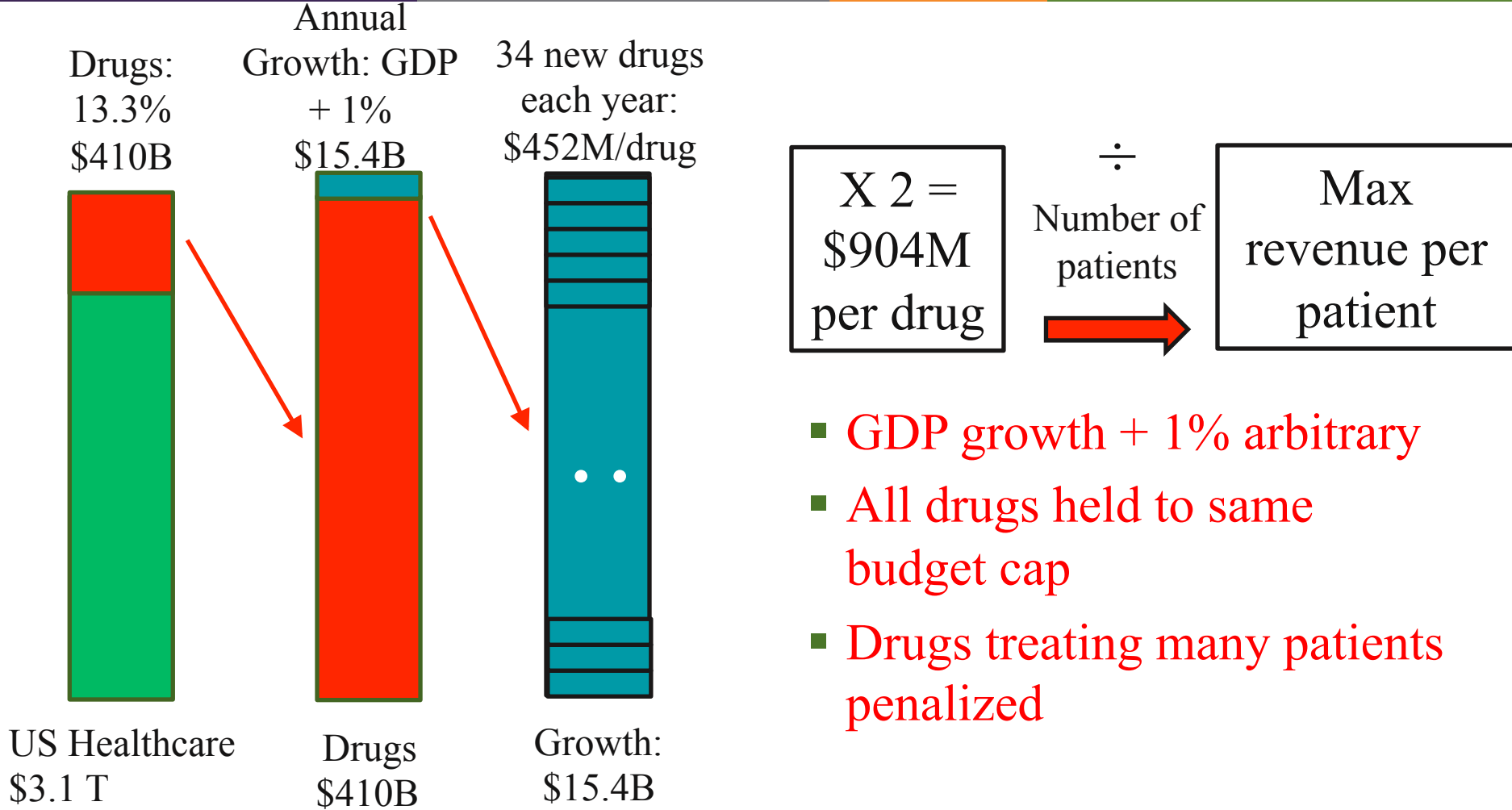
	ACA/AHA	ASCO	ICER	Sloan Kettering	NCCN
Clinical benefit	X	X	X	X	X
Toxicity / safety		X	X	X	X
Treatment novelty				X	
Condition rarity and condition burden				X	
Affordability			X		X
Cost effectiveness	X		X		

Context/ Perspective	Clinical Treatment Guidelines	Shared Decision-Making	Coverage & Payment	Shared Decision-Making & Pricing	Shared Decision-Making
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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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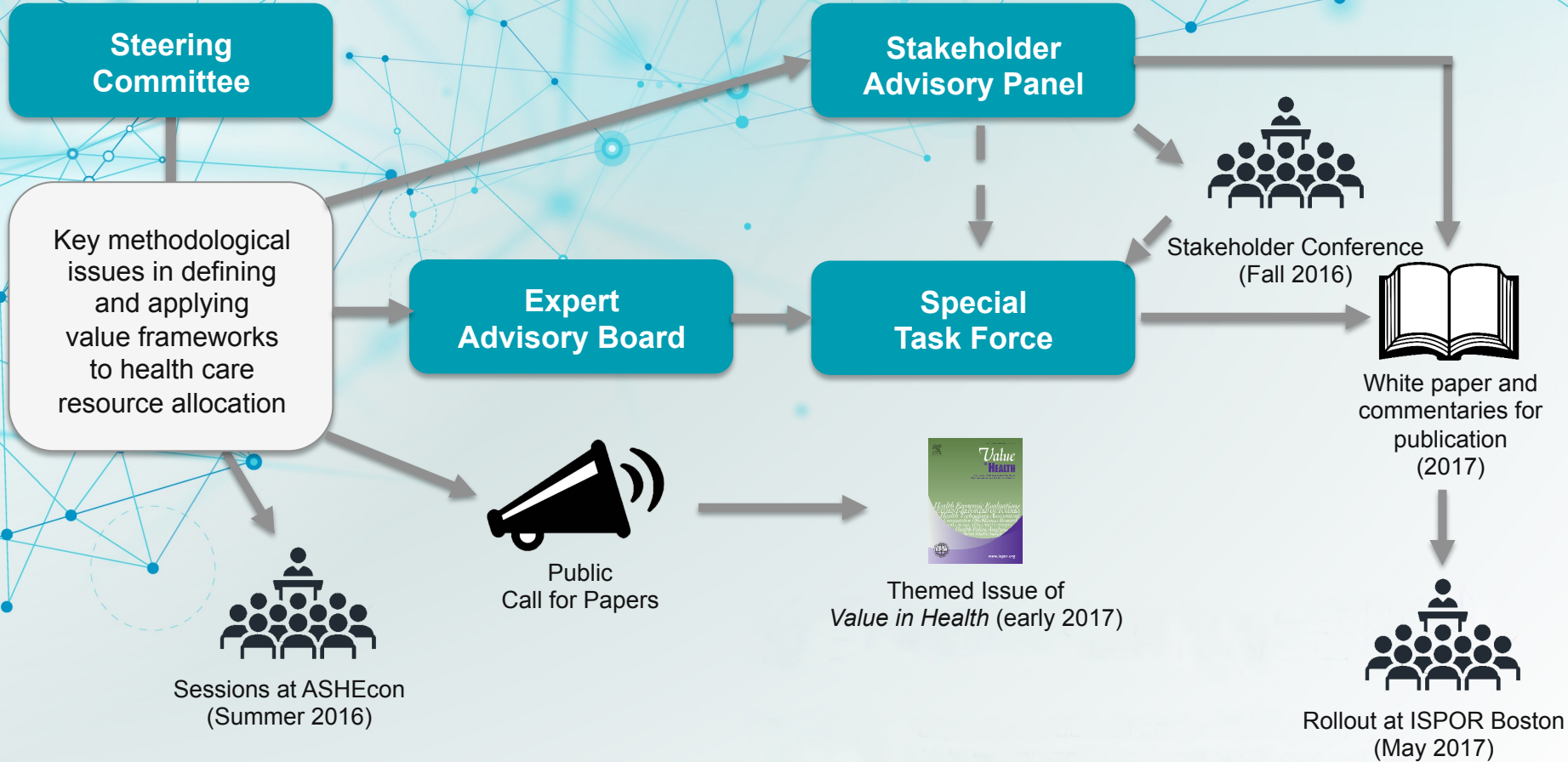
Background: Motivation

- **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.

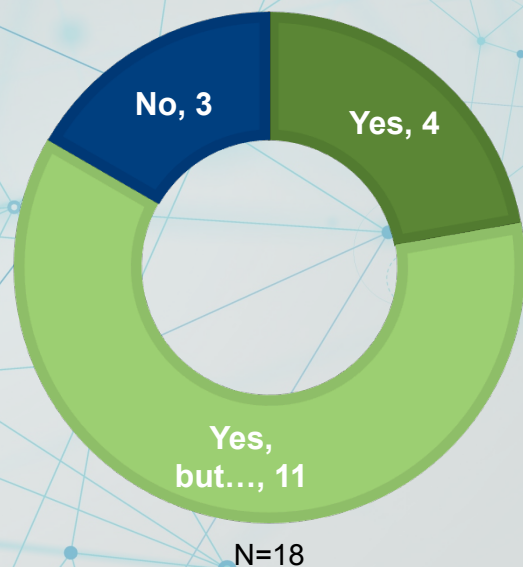
Initiative Aims

- 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



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)

Overall Objective of Special Task Force

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)

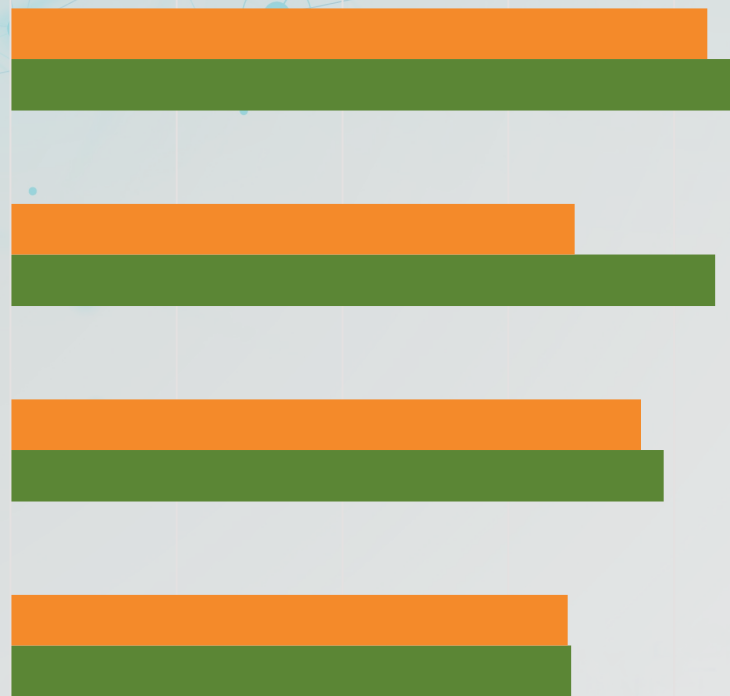
Patient-physician shared decision making

Clinical guidelines (physician as agent for broader clinical/societal considerations)

■ SAP Responses

■ EAB Responses

0 1 2 3 4 5



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

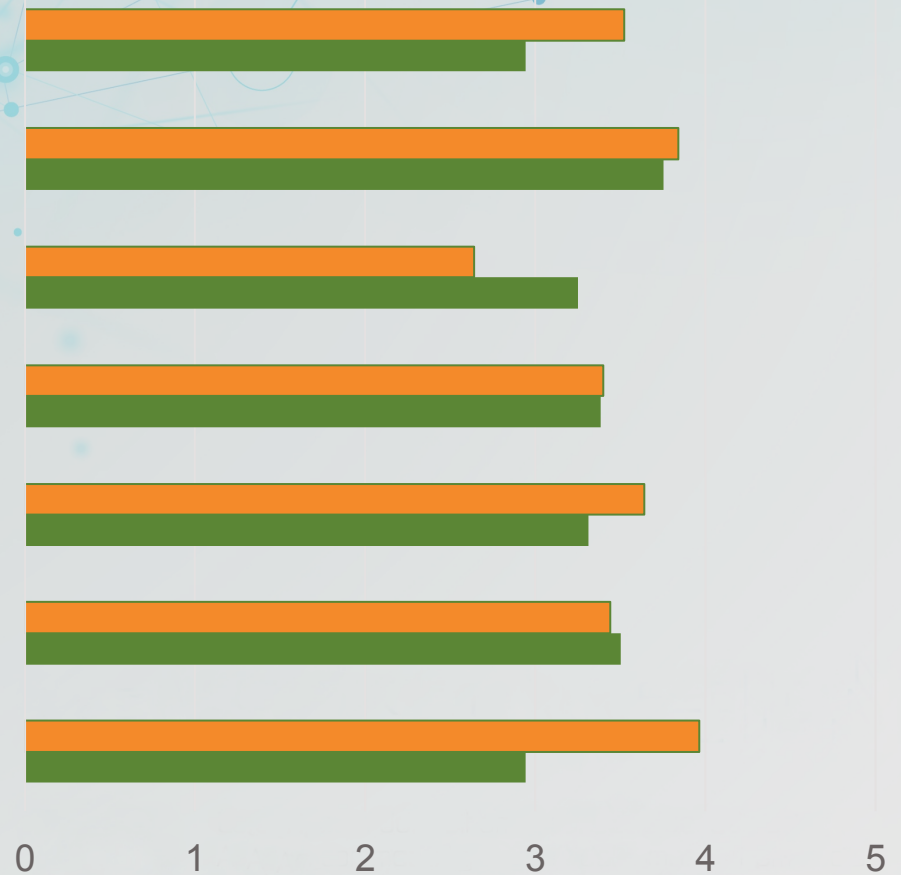
Scientific spillovers

Risk of contagion

Value of reduction in uncertainty due to dx accuracy

Value of hope due to the potential for major treatment benefit

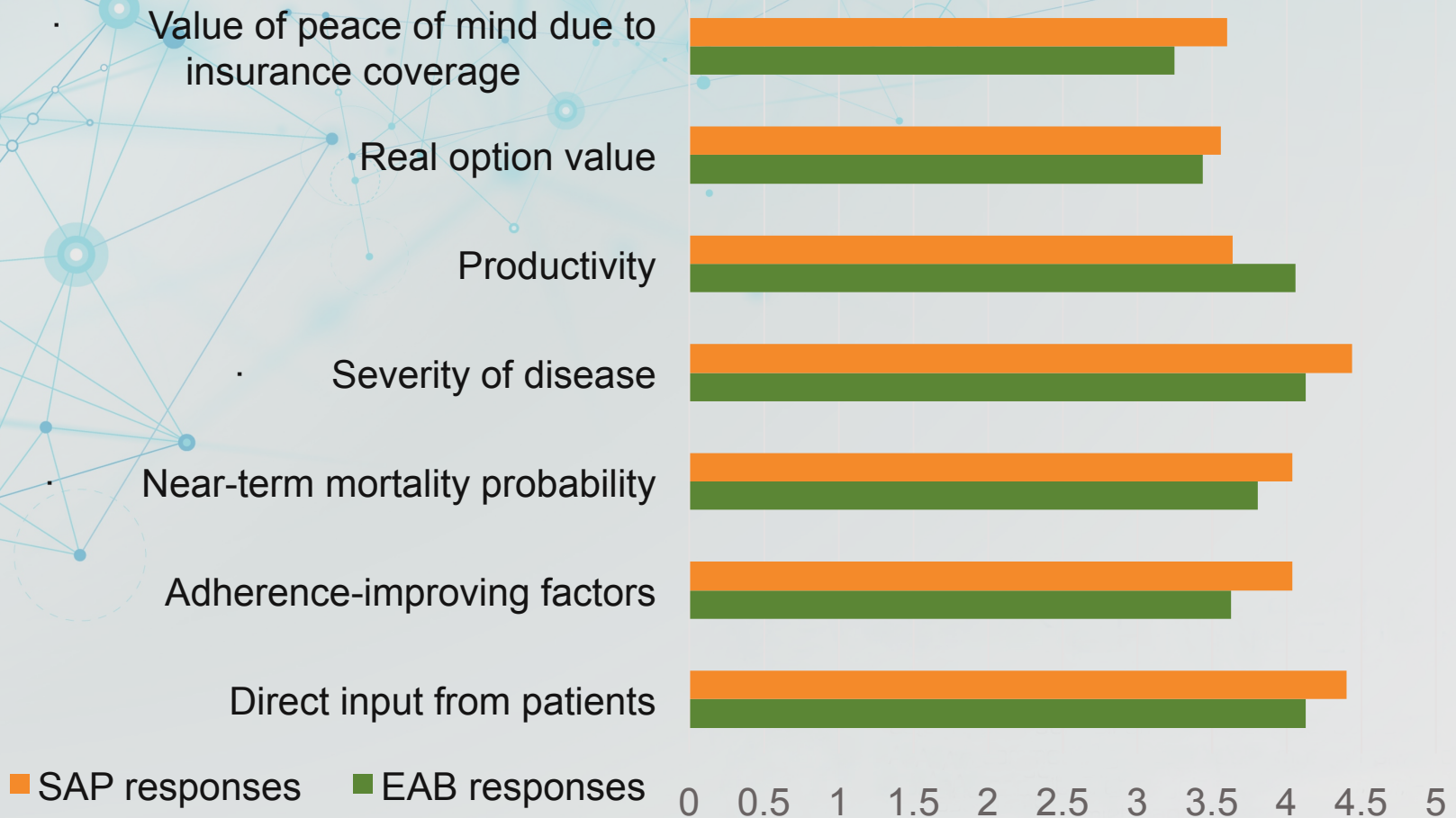
■ SAP responses ■ EAB responses



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)

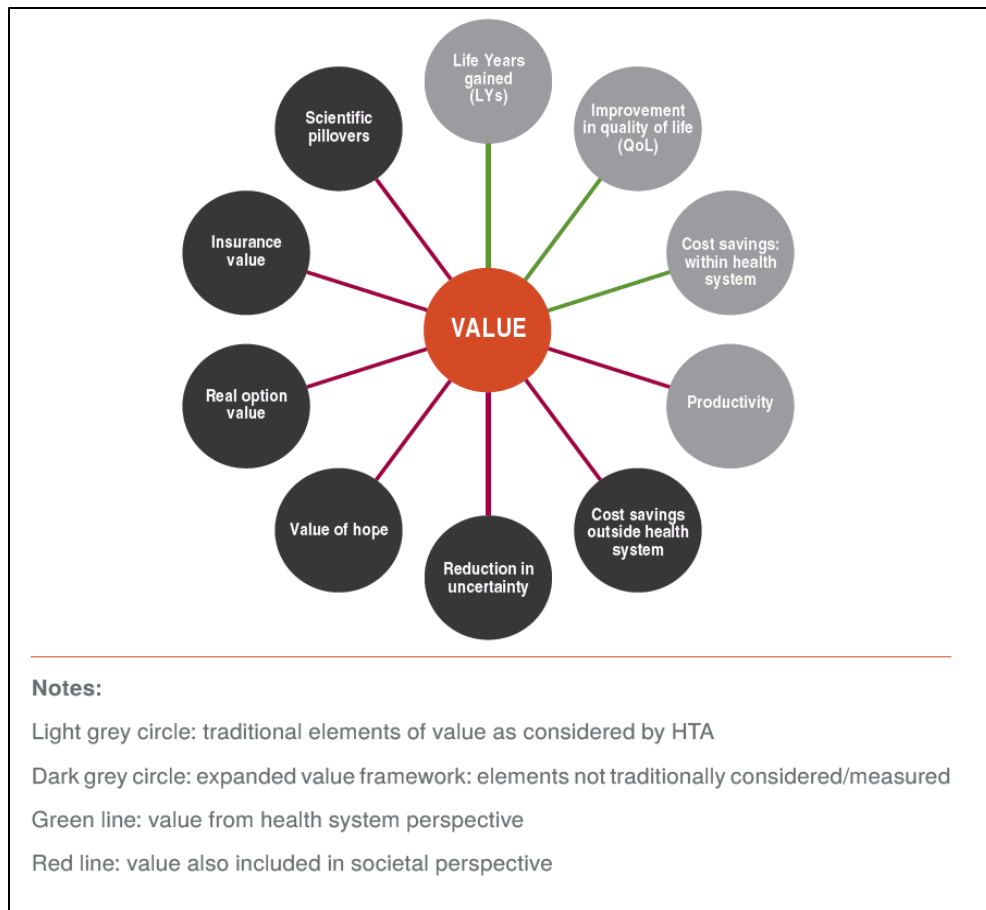


Office of Health Economics Research

EPEMED THE EUROPEAN PERSONALISED MEDICINE ASSOCIATION

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

WHITE PAPER
May 2016



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

	E&W	Australia	Canada	France	Italy	Japan	Sweden
Clinical effectiveness	✓	✓	✓	✓	✓	✓	✓
Cost effectiveness	✓	✓	✓				✓
Alternatives available / unmet need				✓	✓		
Disease severity	EoL			✓	✓		✓
New mode of action						✓	
Paediatric						✓	
Cost savings beyond health care							✓
Productivity							✓

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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