UNIVERSITY of WASHINGTON

Introduction: the Second U.S. Panel Report on CEA: Key Findings and Implications for HTA

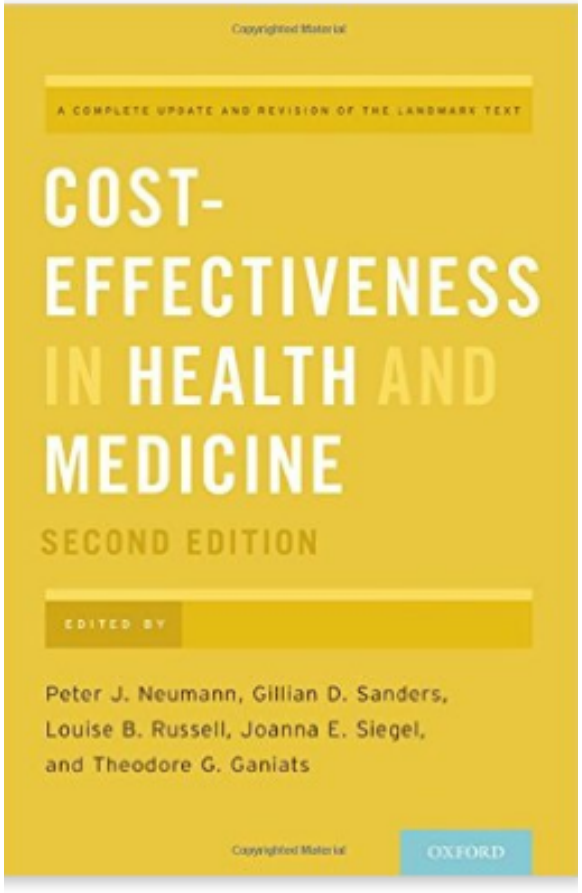
14th International Conference of
Polish Pharmacoeconomics Society –
ISPOR Poland Chapter
Warsaw 2016

Lou Garrison, PhD
Professor, Pharmaceutical Outcomes Research & Policy Program
Department of Pharmacy
Adjunct Professor, Departments of Global Health and Health Services
University of Washington, Seattle, WA, USA
Visiting Senior Fellow, Office of Health Economics, London UK

Agenda

- **Overview of Second Panel Report**
- Emerging value frameworks
- ISPOR Initiative on US Value Frameworks

Second-Panel Volume: Just Released—October 2016



Clinical Review & Education

JAMA | Special Communication

Recommendations for Conduct, Methodological Practices, and Reporting of Cost-effectiveness Analyses

Second Panel on Cost-Effectiveness in Health and Medicine

Gillian D. Sanders, PhD; Peter J. Neumann, ScD; Anirban Basu, PhD; Dan W. Brock, PhD; David Feeny, PhD; Murray Krahn, MD, MSc; Karen M. Kuntz, ScD; David O. Meltzer, MD, PhD; Douglas K. Owens, MD, MS; Lisa A. Prosser, PhD; Joshua A. Salomon, PhD; Mark J. Sculpher, PhD; Thomas A. Trikalinos, MD; Louise B. Russell, PhD; Joanna E. Siegel, ScD; Theodore G. Ganiats, MD

Process

- Formed 2012; non-profit; private effort
- 2 co-chairs, 13 members, and 3 additional members of a leadership group.
- Multi-disciplinary with expertise in the design, conduct, and use of cost-effectiveness analyses.
- Over 3.5 years, the panel developed recommendations by consensus (80% for passage)
- ***Considered each of First Panel recommendations and whether to modify; added some new topic areas—evidence synthesis, decision modeling, ethics***
- Recommendations were reviewed by invited external reviewers and through a public posting process

Key Points

- Kept the concept of the "reference case" to promote quality and comparability
- Recommend two reference cases: "health care sector perspective" and "societal perspective"
- Recommend use of "Impact Inventory"
  - Structured table to define the boundaries of the analysis.

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	☐	☐	
	Health-related quality-of-life effects	☐	☐	
	Other health effects (eg, adverse events and secondary transmissions of infections)	☐	☐	
	Medical costs			
	Paid for by third-party payers	☐	☐	
	Paid for by patients out-of-pocket	☐	☐	
	Future related medical costs (payers and patients)	☐	☐	
	Future unrelated medical costs (payers and patients)	☐	☐	
Informal Health Care Sector				
Health	Patient-time costs	NA	☐	
	Unpaid caregiver-time costs	NA	☐	
	Transportation costs	NA	☐	
Non–Health Care Sectors (with examples of possible items)				
Productivity	Labor market earnings lost	NA	☐	
	Cost of unpaid lost productivity due to illness	NA	☐	
	Cost of uncompensated household production[b]	NA	☐	
Consumption	Future consumption unrelated to health	NA	☐	
Social Services	Cost of social services as part of intervention	NA	☐	
Legal or Criminal Justice	Number of crimes related to intervention	NA	☐	
	Cost of crimes related to intervention	NA	☐	
Education	Impact of intervention on educational achievement of population	NA	☐	
Housing	Cost of intervention on home improvements (eg, removing lead paint)	NA	☐	
Environment	Production of toxic waste pollution by intervention	NA	☐	
Other (specify)	Other impacts	NA	☐	

Source: Sanders, Neumann, et al., 2016

Impact Inventory—Formal Health Sector

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	☐	☐	
	Health-related quality-of-life effects	☐	☐	
	Other health effects (eg, adverse events and secondary transmissions of infections)	☐	☐	
	Medical costs			
	Paid for by third-party payers	☐	☐	
	Paid for by patients out-of-pocket	☐	☐	
	Future related medical costs (payers and patients)	☐	☐	
	Future unrelated medical costs (payers and patients)	☐	☐	

Source: Sanders, Neumann, et al., 2016

Impact Inventory—Informal and Non-Health

Informal Health Care Sector				
Health	Patient-time costs	NA	☐	
	Unpaid caregiver-time costs	NA	☐	
	Transportation costs	NA	☐	
Non–Health Care Sectors (with examples of possible items)				
Productivity	Labor market earnings lost	NA	☐	
	Cost of unpaid lost productivity due to illness	NA	☐	
	Cost of uncompensated household production[b]	NA	☐	
Consumption	Future consumption unrelated to health	NA	☐	
Social Services	Cost of social services as part of intervention	NA	☐	
Legal or Criminal Justice	Number of crimes related to intervention	NA	☐	
	Cost of crimes related to intervention	NA	☐	
Education	Impact of intervention on educational achievement of population	NA	☐	
Housing	Cost of intervention on home improvements (eg, removing lead paint)	NA	☐	
Environment	Production of toxic waste pollution by intervention	NA	☐	
Other (specify)	Other impacts	NA	☐	

Source: Sanders, Neumann, et al., 2016

Second Panel--Key Recommendations

1. Reference Cases and Perspectives
   - Do Reference Case from both "health care sector perspective" and the "societal perspective."
2. Health Care Sector Reference Case
   - Measure health effects in QALYs; summarize as ICER; NMB okay; vary threshold
   - Consider current and **future—related and unrelated--** medical costs
3. Societal Reference Case
   - 3A. Include Impact inventory
   - 3B. Attempt to quantify and value non-health component
   - 3C. Present disaggregated but can combine with ICER if possible

Second Panel--Key Recommendations

4. Reporting Reference Cases and Other Perspectives
   - 4A. State perspective clearly
   - 4B. Present other perspectives
   - 4c. Importance of transparence and sensitivity

Table 1. Cost Components Included in the 2 Recommended Reference Case Perspectives

Cost Component	Reference Case Perspective	
	Health Care	Societal
Formal Health Care Sector[a]		
Costs paid by third-party payers	Yes	Yes
Costs paid out-of-pocket by patients	Yes	Yes
Informal Health Care Sector		
Patient-time costs	No	Yes
Unpaid caregiver-time costs	No	Yes
Transportation costs	No	Yes
Non–Health Care Sectors		
Productivity	No	Yes
Consumption	No	Yes
Social services	No	Yes
Legal or criminal justice	No	Yes
Education	No	Yes
Housing	No	Yes
Environment	No	Yes
Other (eg, friction costs)	No	Yes

[a] Includes current and future costs related and unrelated to the condition under consideration.

Second Panel—Selected Other Recommendations

- Generally follow standard practices with respect to time horizon, costing, discounting, reporting checklist, etc.

Preference Measurement:

- "**Community preferences for health states are the most appropriate** ones for use in the Reference Case analyses. In general, we recommend the use of generic preference based measures such as the EuroQol 5D (EQ-5D), Health Utilities Index (HUI), Short Form 6D (SF-6D), and Quality of Well-Being (QWB). But we also noted that there are situations in which using patient preferences would be preferable." *[emphasis added]*

Source: Sanders, Neumann, et al., 2016

Second Panel—Selected Other Recommendations

Future Costs—Related and Unrelated:

- "The new recommendations also suggest **inclusion of future costs** (ie, that cost-effectiveness analyses account for **related or unrelated health care costs** that occur during the additional life-years produced by an intervention). The original panel discussed this issue but did not reach consensus (noting that analysts could use their discretion) due to the lack of a developed theoretical basis for including future costs at the time of its report." *[emphasis added]*

Source: Sanders, Neumann, et al., 2016

Second Panel—Selected Other Recommendations

Effects on Productivity:

- "In a departure from the original panel, the Second Panel observes that, in general, **effects on productivity are unlikely to have been captured by most preference-based measures**, and that evidence is not definitive that the effects of morbidity on leisure are necessarily reflected in the utility scores or quality-of-life weights. Therefore, it is recommended that the productivity consequences related to changes in health status be reflected in the numerator of cost-effectiveness ratios for reference case analyses conducted under the societal perspective. . ." *[emphasis added]*

Source: Sanders, Neumann, et al., 2016

Second Panel—Concluding Observation

- "Experience shows that when policymakers have incorporated cost-effectiveness analysis into decision-making processes, **they have not applied it as the sole decision criterion. In practice, multiple factors are brought to bear** on resource allocation decisions. Cost-effectiveness is only 1 element among many, including patient's expectations; legal, ethical, equity, cultural, and political concerns; and pragmatic issues of logistics and feasibility." *[emphasis added]*

Source: Sanders, Neumann, et al., 2016

Key Areas for Future Research

"(1) the use of **multi-criteria decision analysis** and group decision making;

(2) the use of cost-effectiveness analysis in **value-based pricing**;

(3) estimation of cost-effectiveness thresholds;

(4) the link between cost-effectiveness analysis and **incentives for innovation**;

(5) the role of cost-effectiveness analysis **within health plans or guideline development**

(6) the **effect of the 2 recommended reference case perspectives** on the cost-effective analysis and its findings." *[emphasis added]*

Source: Sanders, Neumann, et al., 2016

Agenda

- Overview of Second Panel Report
- **Emerging value frameworks**
- ISPOR Initiative on US Value Frameworks

US Drug Value Frameworks

Source: P. Neumann, May 25, 2016

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: Expanding 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

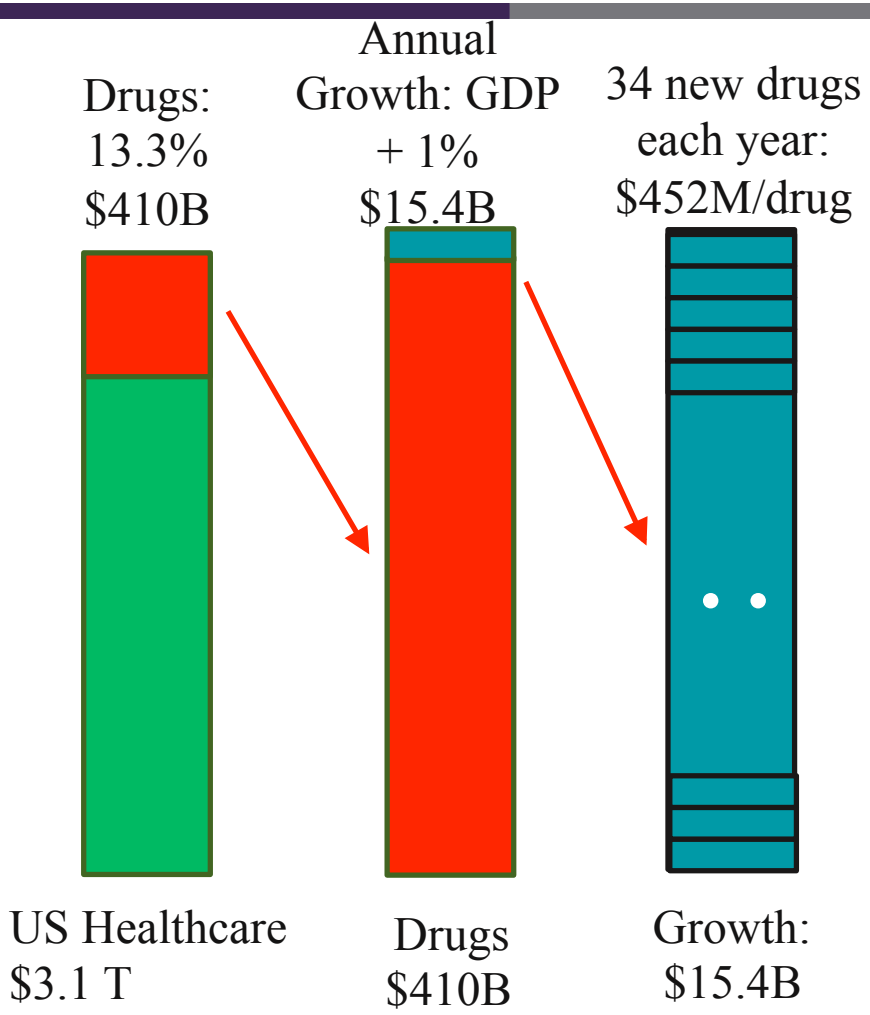
Frameworks use different attributes of value

	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

Source: Adapted from P. Neumann, May 25, 2016

Budget impact: ICER--["Affordability"]



X 2 = $904M per drug

÷ Number of patients

Max revenue per patient

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

Source: P. Neumann, May 25, 2016

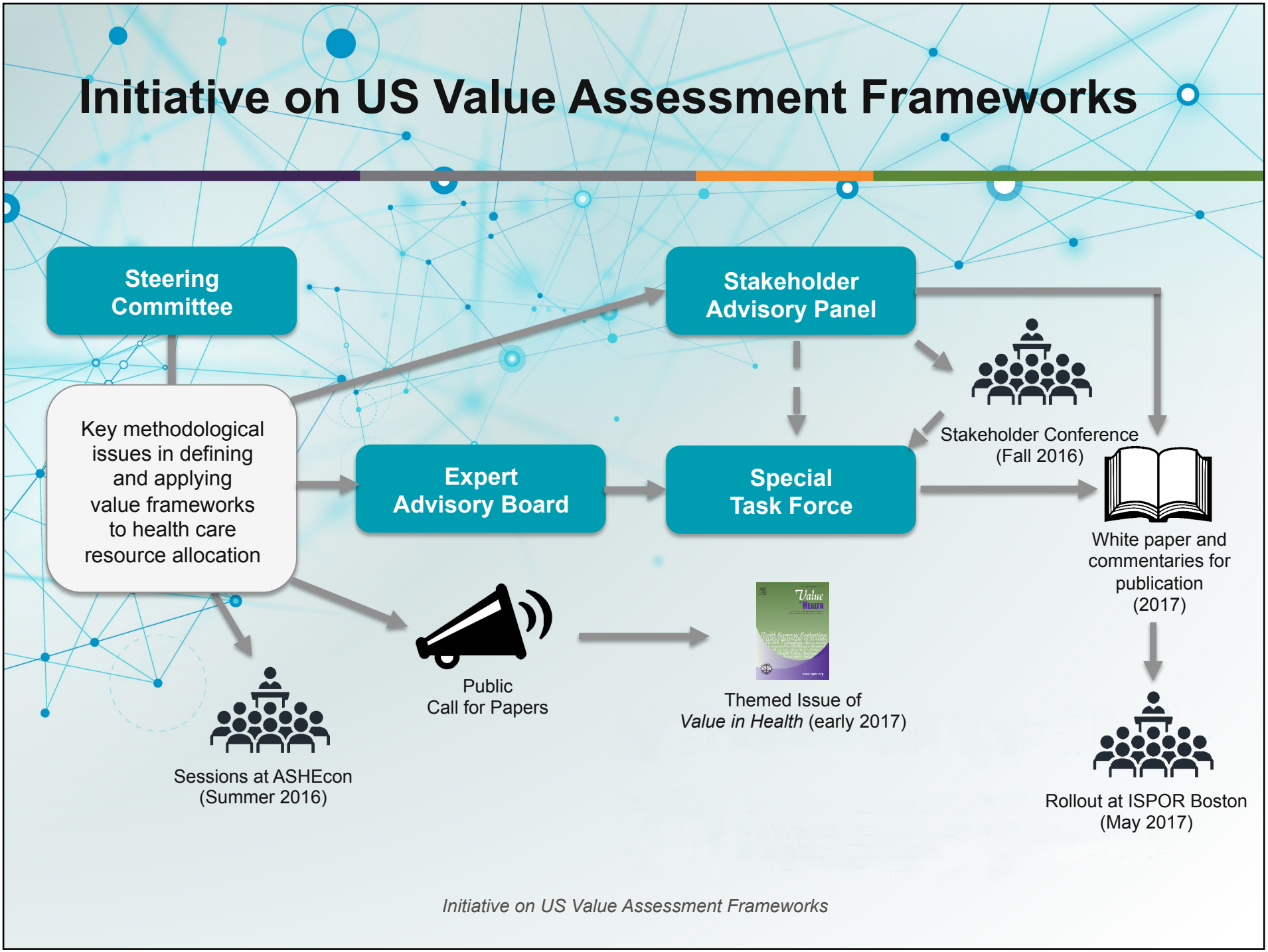
Agenda

- Overview of Second Panel Report
- Emerging value frameworks
- **ISPOR Initiative on US Value Frameworks**

Background: Motivation

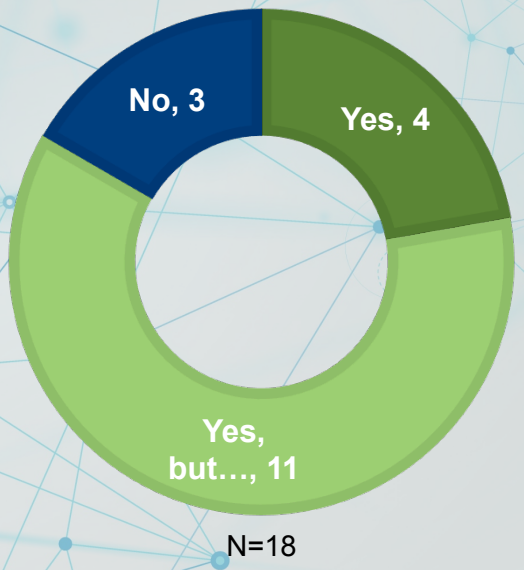
- **In the last few years, a number of value assessment frameworks have been developed** as the health care system has moved toward a **value-driven approach** that focuses on evaluating therapeutic options based on health outcomes, value to the patient, and effectiveness compared with other potential 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 on US Value Assessment Frameworks



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:

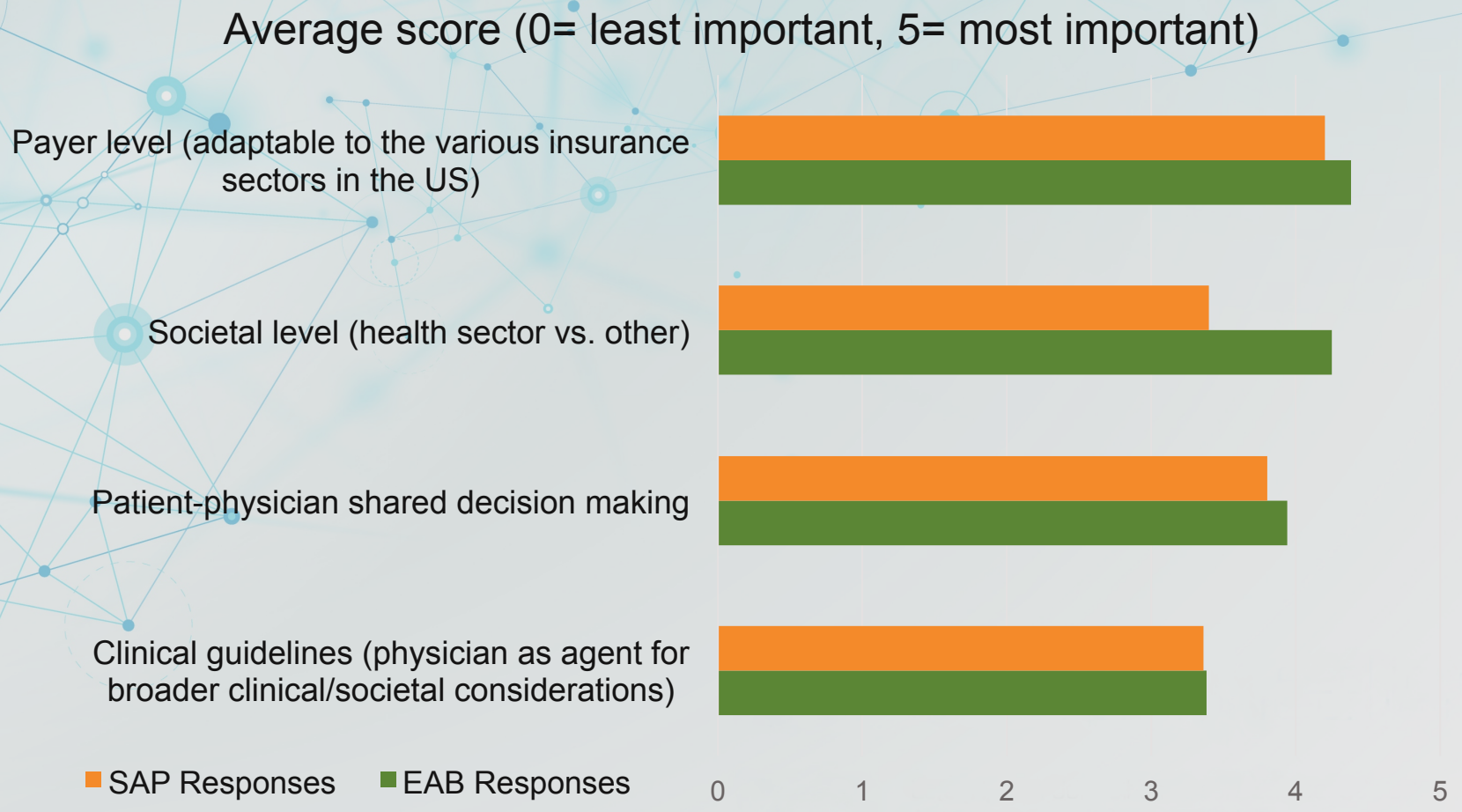
1. CUA contains limitations or is incomplete (n=5)
2. CUA is one of many possible approaches (n=3)
3. The approach must utilize appropriate measures (n=3)

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)

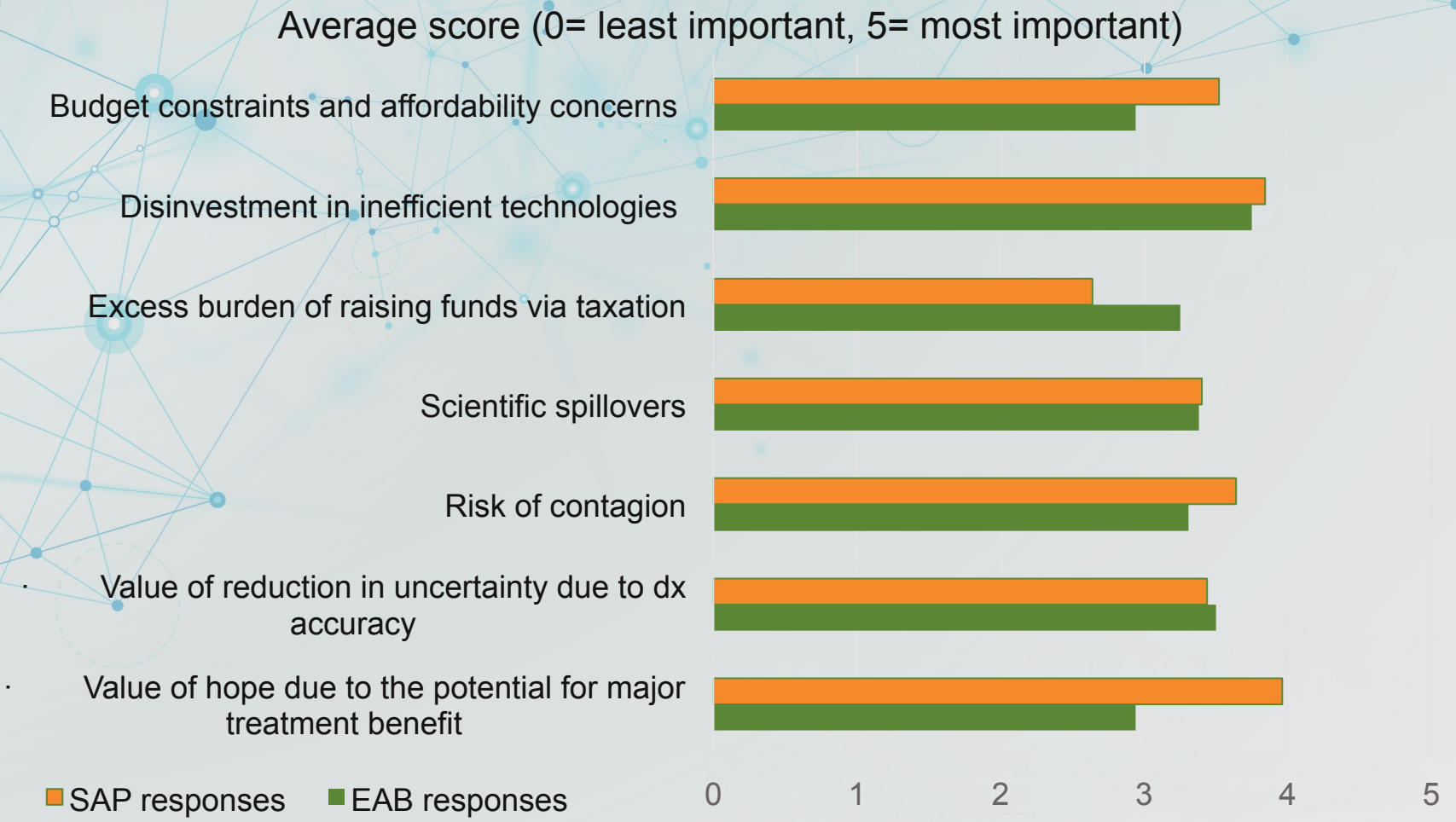
EAB and SAP Survey Question:

Which of the following decision-making contexts are the most important for the STF to consider?



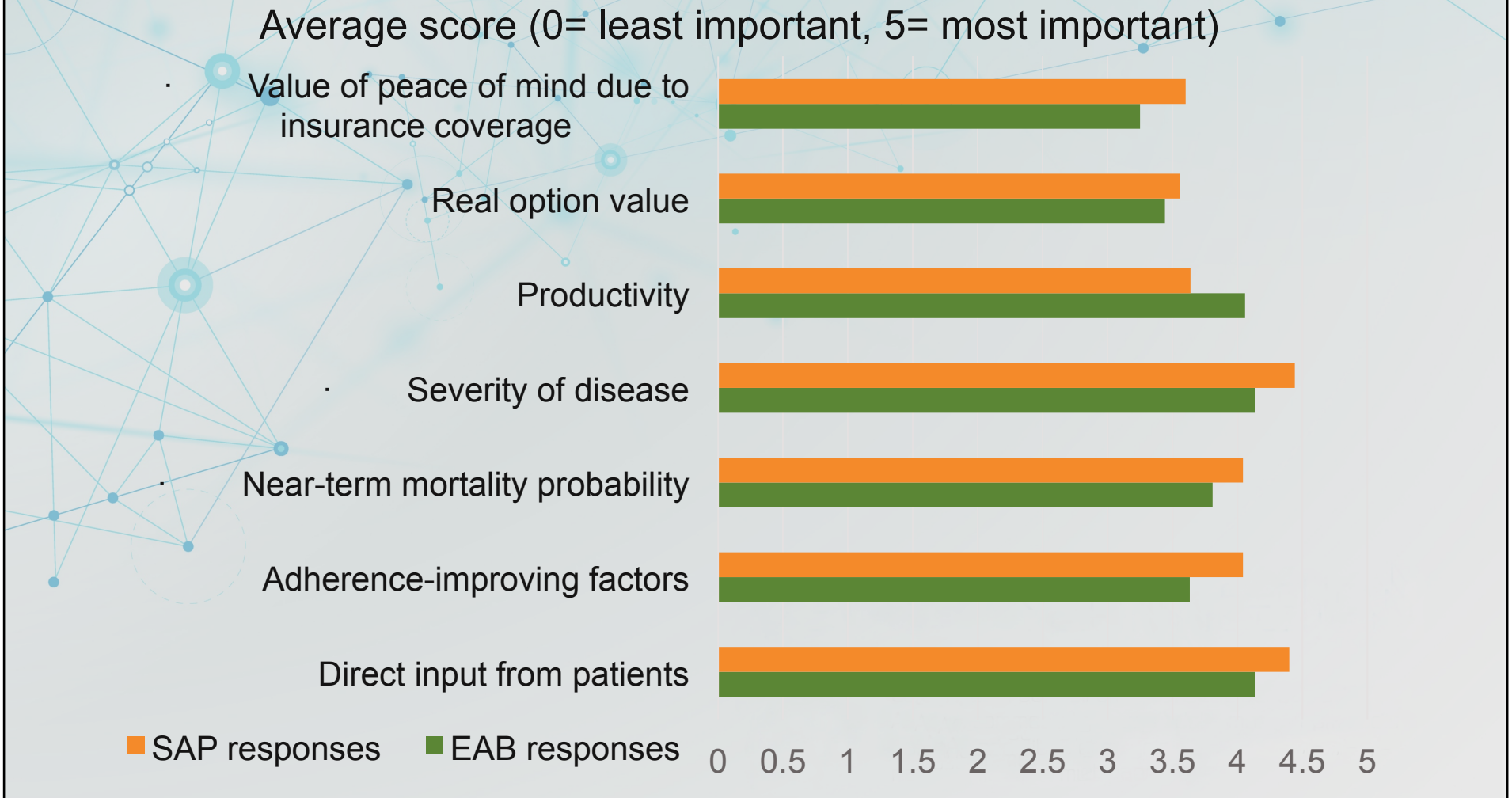
EAB & SAP Survey Question:

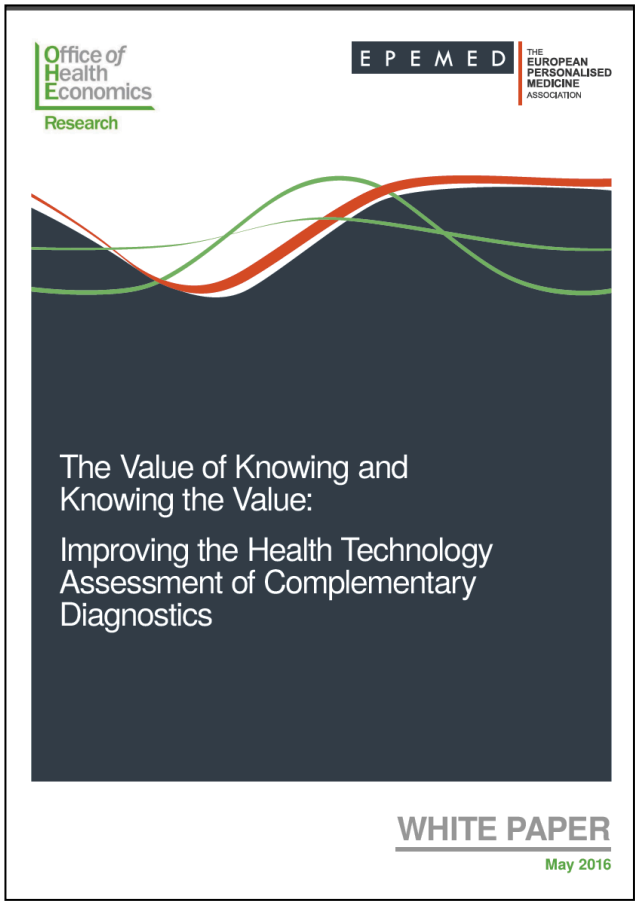
Which of the following potential elements of value are the most important for the STF to consider? (part 1)

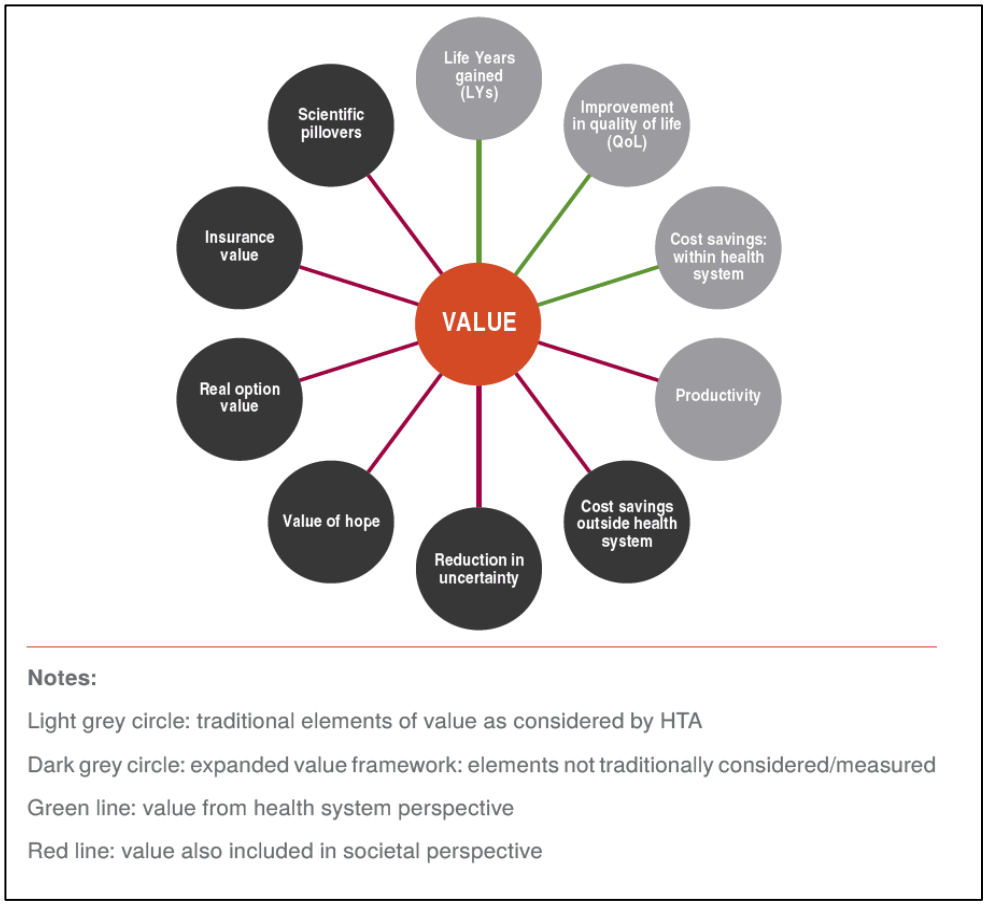


EAB & SAP Survey Question:

Which of the following potential elements of value are the most important for the STF to consider? (part 2)







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?

Thanks for your attention

lgarrisn@uw.edu