

Fake or Novel Elements of Value: Approach of the ISPOR Special Task Force on U.S. Value Frameworks

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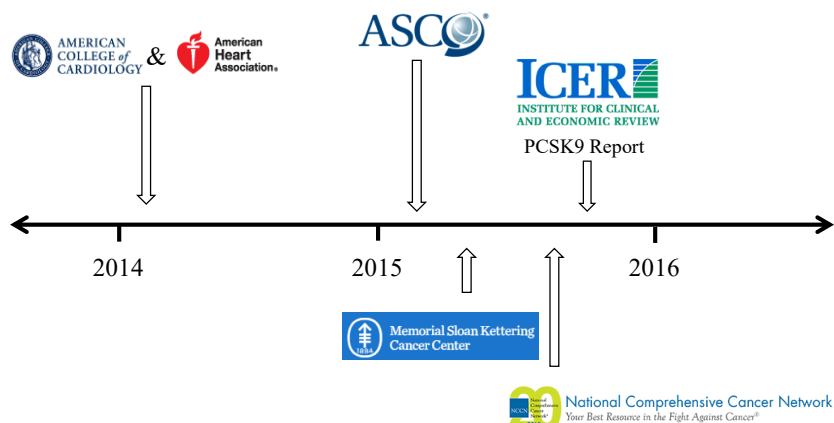
VIEWPOINTS

Reflections on the ISPOR Special Task Force on U.S. Value Frameworks: Implications of a Health Economics Approach for Managed Care Pharmacy

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Key U.S. Value Frameworks Considered by ISPOR STF



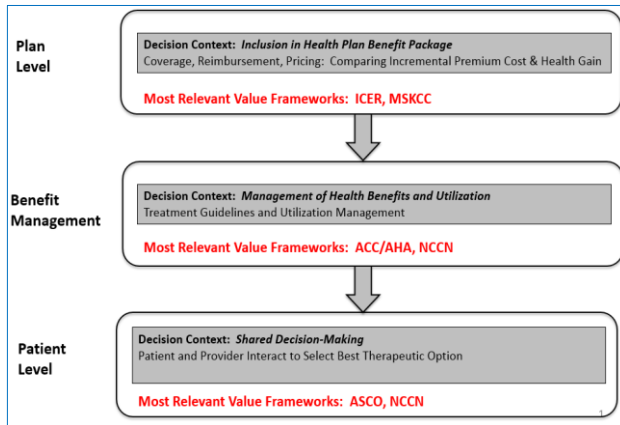
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TABLE 1 ISPOR Special Task Force Recommendations

Primary Recommendations	Key Points
I. Be explicit about decision context and perspective in value assessment frameworks.	<ul style="list-style-type: none"> No single value assessment framework captures everything. For societal and health plan resource allocation decisions (coverage/reimbursement), the perspective should reflect those who pay for care (e.g., enrollees, employees, taxpayers). Well-designed patient-level frameworks can help guide shared decision making for treatment choices.
II. Base health plan coverage and reimbursement decisions on an evaluation of the incremental costs and benefits of health care technologies as is provided by cost-effectiveness analysis.	<ul style="list-style-type: none"> Cost-per-QALY analyses have strengths and limitations. Frameworks that focus on coverage/reimbursement should consider cost per QALY, as a starting point. Consider elements not normally included in CEAs (e.g., severity of illness, equity, risk protection) but more research is needed.
III. Develop value thresholds to serve as an important input to help guide coverage and reimbursement decisions.	<ul style="list-style-type: none"> Assess value for money and compare to value threshold, allowing for other factors. In the United States, different payers could use different thresholds. Equity and severity of illness considerations may alter thresholds.
IV. Manage budget constraints and affordability based on cost-effectiveness principles.	<ul style="list-style-type: none"> Budget impact analysis is not integral to value assessment. Budget constraints and affordability can be addressed using current tools, e.g., delay or cost-effectiveness ratios. Over time, availability of new cost-effective technologies may affect overall spending.
V. Test and consider using structured deliberative processes for health plan coverage and reimbursement decisions.	<ul style="list-style-type: none"> No existing method of aggregation is perfect. Pragmatic approaches are needed. Deliberative process is useful and important. Deliberative processes should consider explicit ACEA and MCDA.^a MCDA—with appropriate weights—could be helpful for patients choosing treatments.
VI. Explore and test novel elements of benefit to improve value measures that reflect the perspectives of plan members and patients.	<ul style="list-style-type: none"> Develop more comprehensive economic evaluation. More research needed on ACEA and MCDA. Payers are agents for patients. Patient experience is central.

Observation #1: To understand differences among existing and emerging value assessment frameworks, it is important to distinguish among “perspectives” and among “decision contexts.” [see Recommendation I]

Cascade of Decision Contexts



Perspectives:

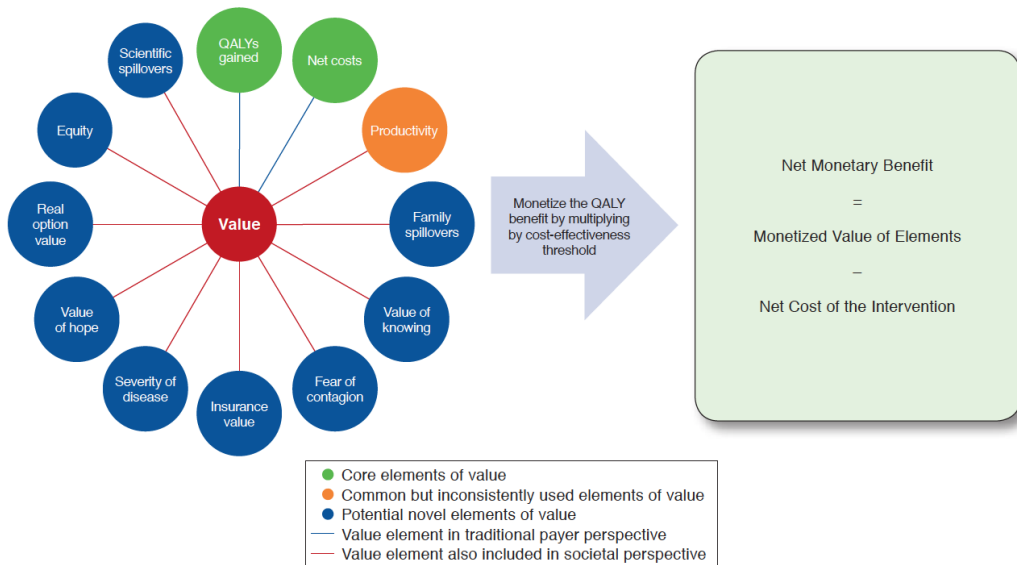
- Societal
- Healthcare sector
- Insurer/health plan
- Provider
- Patient

Second-Panel Volume: Impact Inventory (October 2016)



Figure 1. Impact Inventory Template

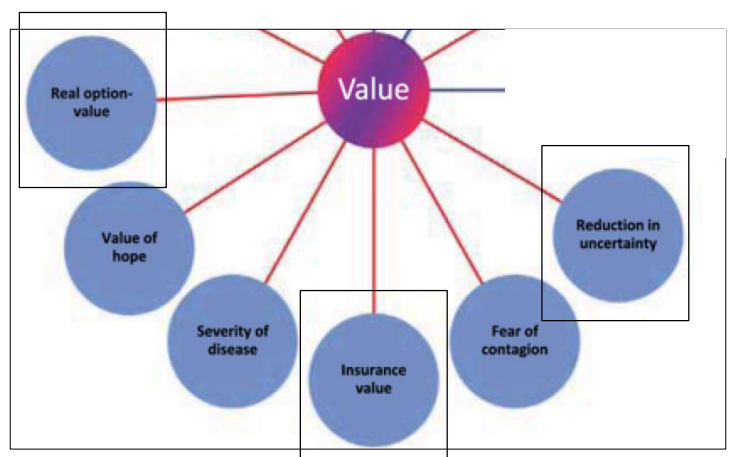
Sector	Type of Impact (list category within each sector with unit of measure if relevant) ^a	Included in This Reference Case Analysis From...Perspective?		Notes on Sources of Evidence
		Health Care Sector	Societal	
Formal Health Care Sector				
Health	Health outcomes (effects)			
	Longevity effects	<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"/>	
Informal Health Care Sector	Paid for by patients out-of-pocket	<input type="checkbox"/>	<input type="checkbox"/>	
	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"/>	
	Unpaid caregiver-time costs	NA	<input type="checkbox"/>	
Health	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"/>	
Consumption	Cost of uncompensated household production ^b	NA	<input type="checkbox"/>	
	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"/>	



Adapted from: Lakdawalla DN, Doshi JA, Garrison LP Jr, Phelps CE, Basu A, Danzon PM. Defining elements of value in health care—a health economics approach: an ISPOR Special Task Force Report [3], p. 132. Reprinted with permission.
MCDA= multicriteria decision analysis; QALY= quality-adjusted life-year.

Potential Elements of Value: Elements Related to Uncertainty (1)

- **Insurance value**
 - Financial risk protection AND
 - Health risk protection
 - Can adjust for severity and rarity;
 - In Extended Cost-Effectiveness Analysis
- **Reduction in uncertainty due to Dx test (also called “Value of Knowing”)**
 - Text-drug combination is more valuable
 - Value in prognosis
- **Real option value**
 - Investing in a life-extending treatment provides more value in disease area with more promising pipeline



Potential Elements of Value: Elements Related to Uncertainty (2)

- **Value of hope**

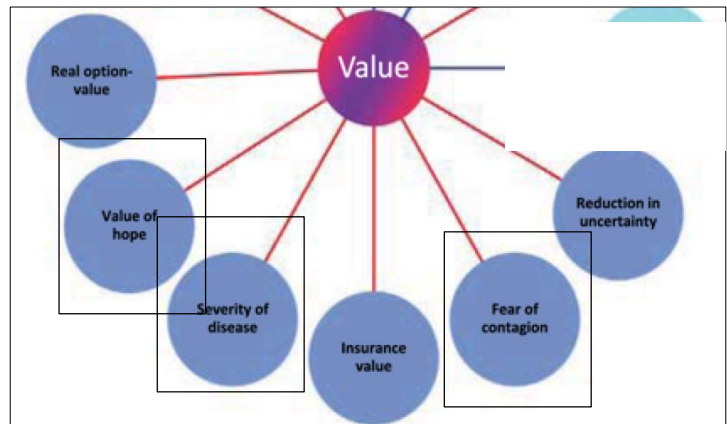
- Many patients are willing to sacrifice some life expectancy for the chance for a cure.

- **Severity of disease**

- Greater willingness to pay for more severe diseases (beyond the QALY loss)

- **Fear of contagion**

- A psychic externality due to worry about spread of infectious disease (e.g., Zika virus)



Studies Measuring Insurance Value

Study	Context	Method	Impact Above Convention ICER
Verguet et al. 2013	Rotavirus-India (I) & Ethiopia (E)	Dynamic CEA Modeling	FRP of \$16k and \$8K for I and E per 1 mil. Largest FRP in lowest income group.
Verguet et al. 2015	TB in India	Universal public finance model	Per mil., insurance value is \$9,000 and 80% to two bottom quintiles.
Shih et al., 2016	Multiple sclerosis in U.S.	Parameterized utility function	33%
Lakdawalla et al., 2017	General U.S. population	Numerical exercise with a parameterized utility function	38% to 62%; Physical insurance value exceeds financial value

Studies Measuring Real Option Value

Study	Context	Method	Impact
Sanchez et al., 2012	Imatinib for chronic myeloid leukemia in U.S.	Projection of mortality trends	9% of conventional survival benefit
Snider et al., 2017	Nivolumab for renal cell carcinoma and lung cancer in U.S.	Projection of mortality trends	5-18% of conventional survival benefit
Li et al., 2018	Ipilimumab for metastatic melanoma in U.S.	Historical interrupted time series	Affected treatment patterns
Li et al., 2018	Ipilimumab for metastatic melanoma in U.S.	Projection of mortality trends and new drug approvals and economic modeling	Incremental QALY gained increased by 5-8% and ICER decreased by 0-2%

Studies Measuring Value of Hope

Study	Context	Method	Impact
Philipson et al., 2010	End of life/terminal care	Economic estimation (ex post)	Willingness to trade gains in average survival for greater variance
Lakdawalla et al., 2012	Treatments for metastatic melanoma and metastatic breast cancer in U.S.	Discrete Choice/Contingent Valuation	WTP \$35,000 for a 1 SD increase in survival
Shafrin et al., 2017	Treatments for advanced stage melanoma or lung cancer in U.S.	Patient and physician surveys	Majority of patient prefer higher variance in survival; physicians do not.
Shafrin et al., 2018	Nivolumab for squamous non-small cell lung cancer in U.S.	Economic estimation	0.04 QALY

Studies Measuring Value of Knowing (via Dx)

Study	Context	Method	Impact
Goldman et al, 2013 (Sood et al. 2013 technical analysis)	Dx testing in personalized medicine: RA patients at risk for CV event on Vioxx in U.S.	Population economic modeling	Test generates \$1,284 per patient.
Neumann et al., 2012	Predictive testing for diseases with no prevention	Stated-preference study	\$109 - \$263 per test

Other Potential Novel Elements: Non-QALY and Non-cost-offset

Uncertainty-related:

1. Insurance value
2. Value of hope
3. Value of knowing
4. Real option value
5. Severity of disease
6. Fear of contagion

Non-Uncertainty-related:

1. Equity
2. Scientific spillovers
3. Adherence
4. Family spillovers
5. Health system readiness
6. Impact on non-health sectors

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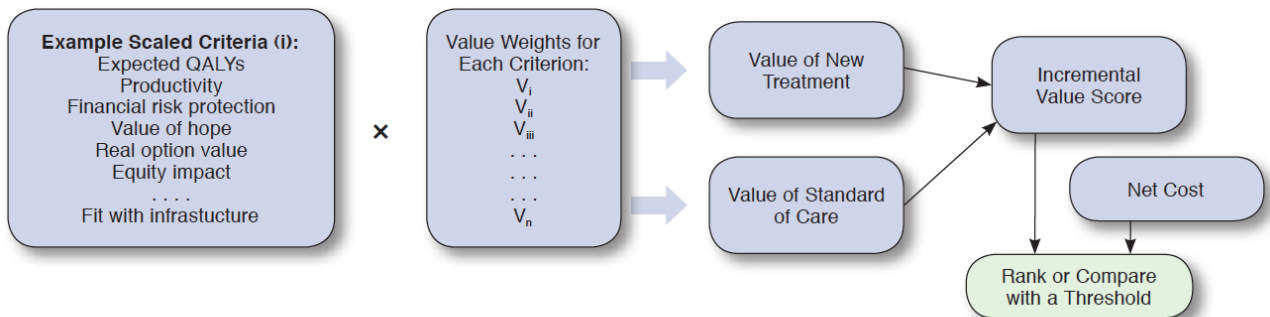
NATIONAL BUREAU OF ECONOMIC RESEARCH
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July 2019

“Traditionally, cost-effectiveness analyses have relied on average health outcomes to assess the value of clinical interventions. Yet, focusing on averages overlooks the role of risk and uncertainty in the effects of medical technologies.”

“Our simulations suggest that **ignoring these stochastic components of treatment outcomes can seriously bias estimates of incremental cost-effectiveness**, either in upward or downward direction, depending on how the new technology alters the risk profile of patient outcomes. **Repairing this defect merely requires good estimates of skewness (and possibly kurtosis) measures of clinical outcomes in studies comparing medical interventions**, and incorporation of those parameters into our new model.” *[Emphasis added]*

FIGURE 1 Multicriteria Decision Analysis for Comparison with ACEA Approach

Estimate Value for Each Intervention:



ACEA = augmented cost-effectiveness analysis; QALY = quality-adjusted life-year.

Key Recommendation of STF

Key Recommendation: Our ISPOR STF recommended further methods development and testing of alternative approaches that build on a cost-per-QALY metric, including ACEA and MCDA in support of deliberative processes.

Implications (My view):

1. The QALY is the key criterion and will be the driver under either ACEA or MCDA approaches.
2. More work is needed on both regarding estimating the value associated with the other non-QALY criteria, which can be categorized as those related to uncertainty and those related to other factors.
3. The estimation of the CE threshold will change and need to be adjusted in applying these.
4. Either ACEA or MCDA will need to feed into a deliberative process for formulary/benefit package inclusion.
5. Inclusion of uncertainty-related elements speaks to maximizing “well-being” vs. health.

Thank you!

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