Past Imperfect, Present Tense, Future Conditional:
Where next for value frameworks?

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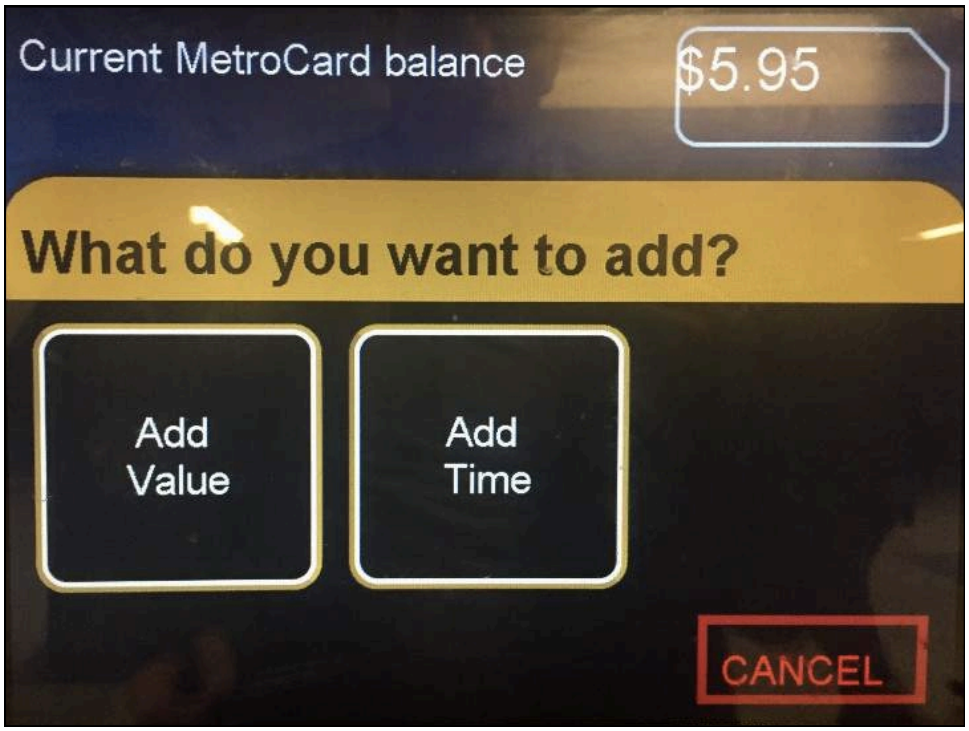

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DIVIDE ET IMPERA: PROTECTING THE GROWTH OF HEALTH CARE INCOMES (COSTS)

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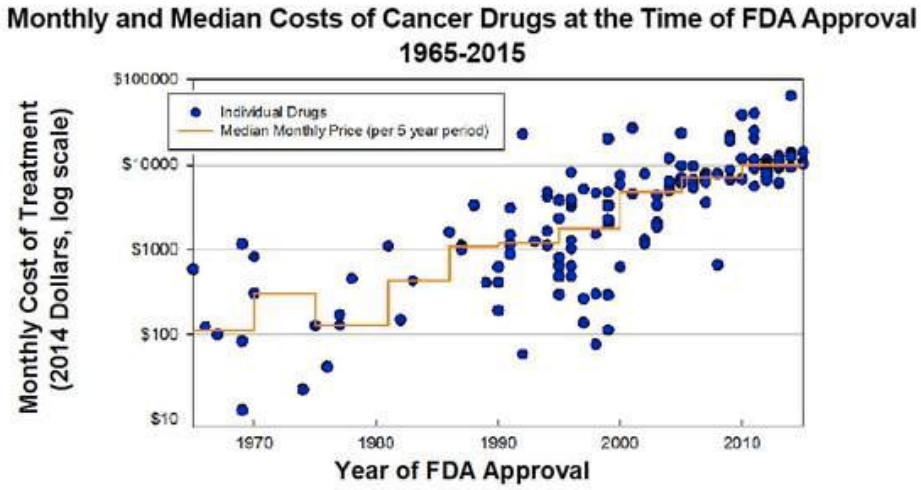
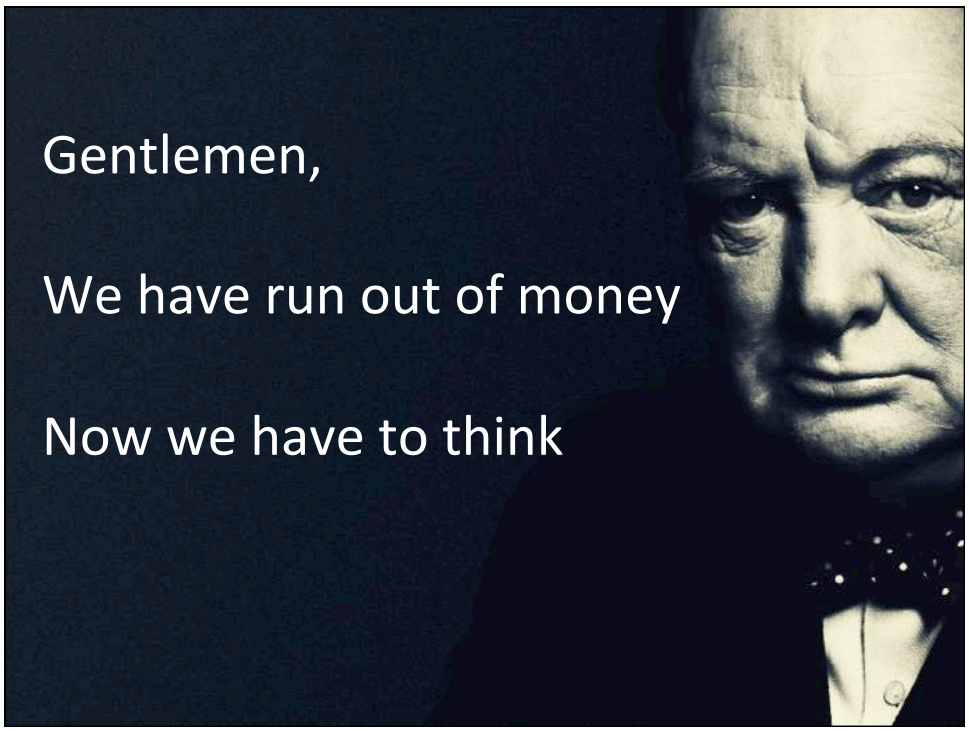
It is proper to preamble an essay of this sort with the observation that anyone who has received health care for a serious illness is likely to agree that, with few exceptions, health care sectors in the USA and elsewhere tend to be staffed with millions of smart and highly trained professionals who sincerely seek to improve the quality of their patients' lives. Their admirable clinical efforts, however, are embedded in a ceaseless struggle over money. That struggle is be the focus of this essay.

Every health care system naturally pursues two distinct goals, as is illustrated in Figure 1, namely (1) enhancing the quality of patients' lives and (2) enhancing the quality of lives of those who provide real resources to the process of health care (Reinhardt, 1987). Patients also play a dual role in this setting, of course. They are both objects of human compassion and biological structures yielding cash flows, which in the USA can be openly traded on the stock exchanges (Reinhardt, 1999). A strictly observed etiquette in public debates on health policy, however, is never to talk openly about the second goal of health care. Any proposal to enhance the second goal therefore must be styled to seem to further the first goal—e.g. 'enhancing the quality of patient care', 'innovating to create value for patients', 'saving lives', and so on. This essay will stray from that tradition.


Monthly and Median Costs of Cancer Drugs at the Time of FDA Approval
1965-2015
Individual Drugs
Median Monthly Price (per 5 year period)
Monthly Cost of Treatment
(2014 Dollars, log scale)
$100000
$10000
$1000
$100
$10
1970
1980
1990
2000
2010
Year of FDA Approval


Gentlemen,
We have run out of money
Now we have to think

ACA, PCORI and cost-per-QALY

"The Patient-Centered Outcomes Research Institute … shall not develop or employ a dollars-per-quality adjusted life year … as a threshold to establish what type of health care is cost effective or recommended."

The Patient Protection and Affordable Care Act, 2010
Neumann & Weinstein, 2010

Therapy Value Frameworks in US

- American College of Cardiology- American Heart Association (ACC-AHA)
- Institute for Clinical and Economic Review (ICER)
- National Comprehensive Cancer Network (NCCN)
- American Society of Clinical Oncology (ASCO)
- Memorial Sloan-Kettering Cancer Center (MSKCC)
- 2nd US Panel on Cost-effectiveness

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PERFORMANCE MEASURES

ACC/AHA Statement on Cost/Value Methodology in Clinical Practice Guidelines and Performance Measures

A Report of the American College of Cardiology/American Heart Association Task Force on Performance Measures and Task Force on Practice Guidelines

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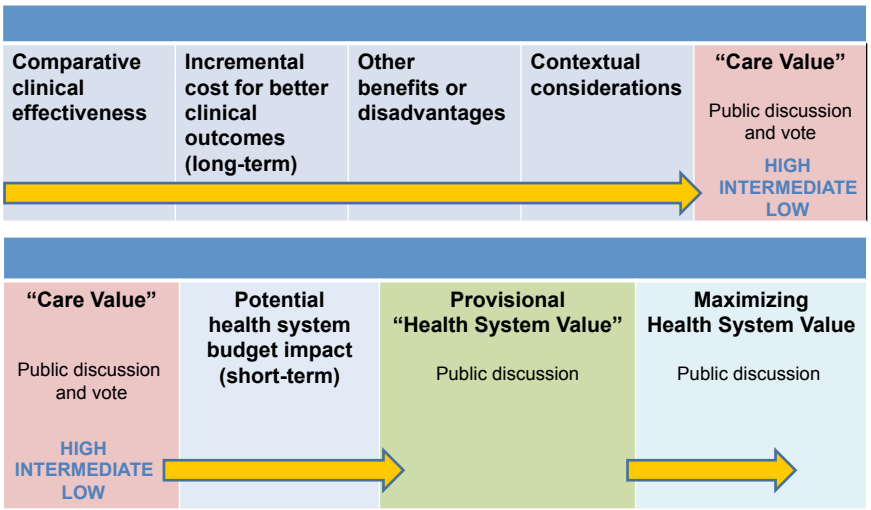
*Writing committee members are required to recuse themselves from voting on sections to which their specific relationships with industry may apply; see Appendix 2 for detailed information.

ACC-AHA Statement

- High value: <$50,000 per QALY
- Low value: >$150,000 per QALY
- Value thresholds could be pegged to GDP or other socially accepted norm
- Supplemented by quality of evidence level

This report stresses that the value category should be only one of several considerations in medical decision making and resource allocation. Providers and society may be willing to pay more for the only effective treatment for a rare disease (e.g., congenital versus adult cardiac care).

ICER Value Assessment Framework



US Panel on Cost-Effectiveness

In 1993, the U.S. Public Health Service convened a panel of 13 non-government scientists and scholars to review the developing field of cost-effectiveness analysis.

Recommendations published in 1996 by OUP

Recommended QALY as output by which to measure value

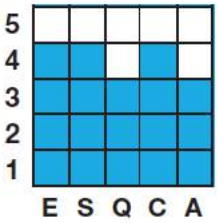
US Panel on Cost-Effectiveness II has just reported 2016

QALYs remain recommended metric

Are cancer frameworks special?

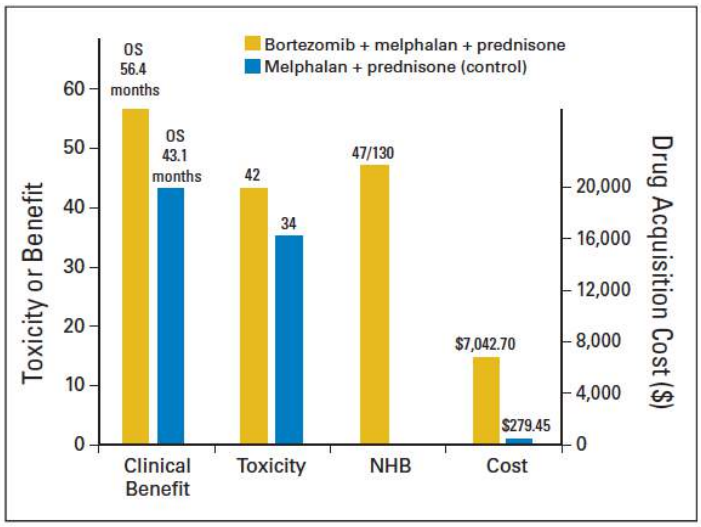
NCCN Evidence Blocks

NCCN EVIDENCE BLOCKS CATEGORIES AND DEFINITIONS

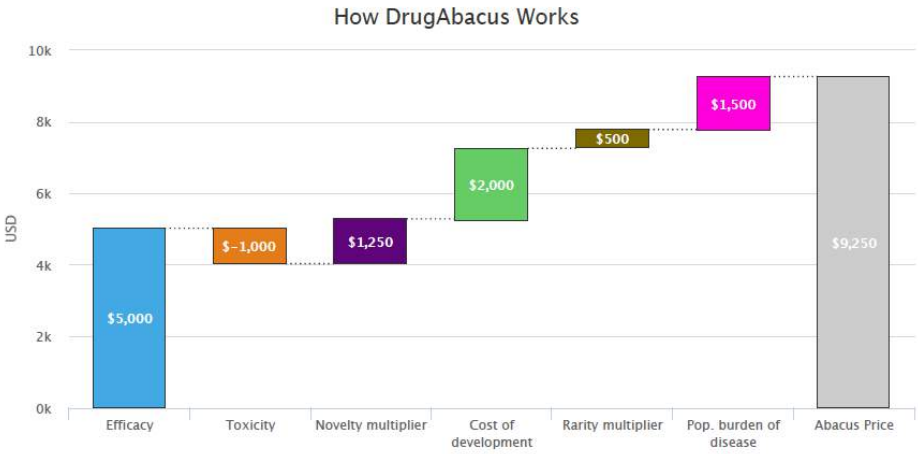


E = Efficacy of Regimen/Agent
S = Safety of Regimen/Agent
Q = Quality of Evidence
C = Consistency of Evidence
A = Affordability of Regimen/Agent

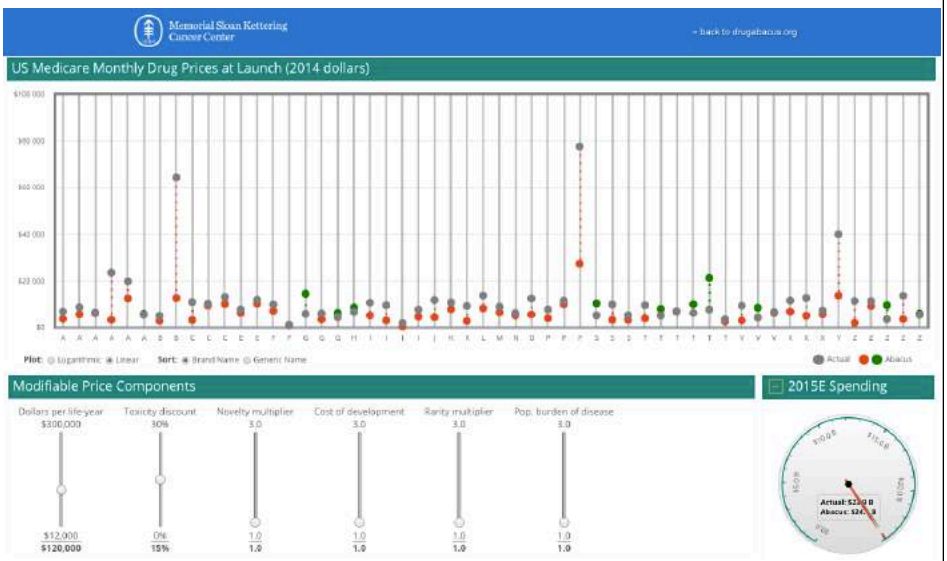
ASCO Value Framework



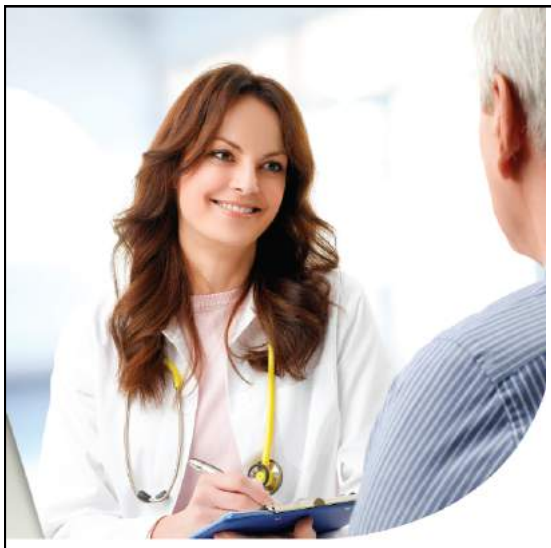
MSKCC Drug Abacus



MSKCC Drug Abacus in action



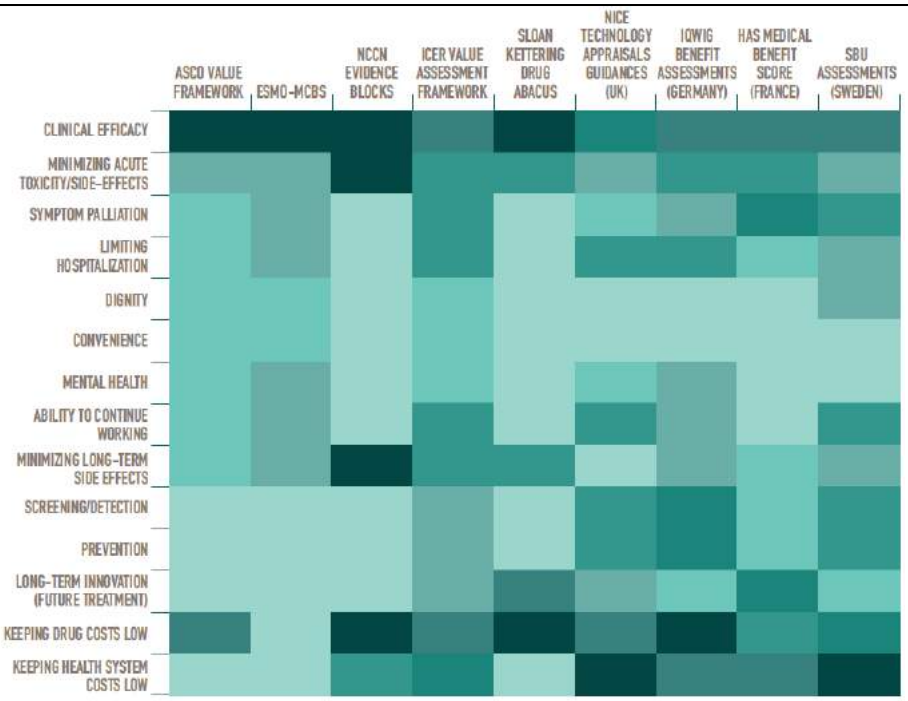
Response from industry?

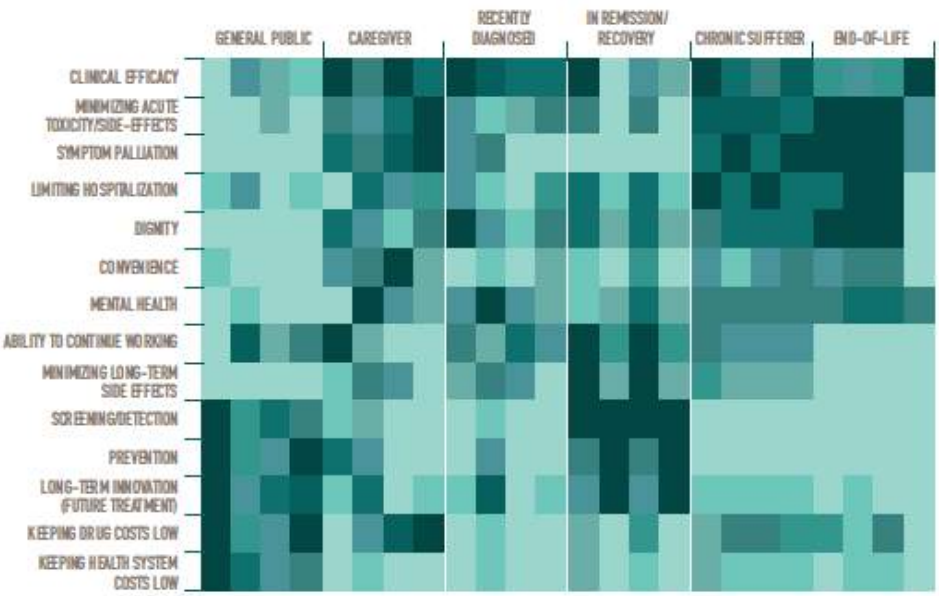


Concept Paper

- Results of a workshop
- Supported and facilitated by Grayling
- Funded by Eli-Lilly

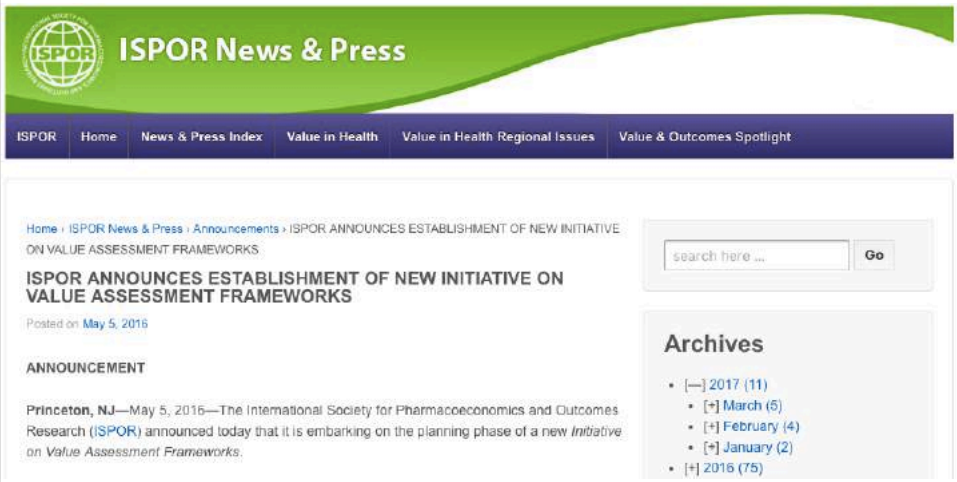
PERSPECTIVES ON VALUE IN CANCER CARE





Disease specific MCDA?

ISPOR / ASHEcon White Paper



ISPOR News & Press

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Home › ISPOR News & Press › Announcements › ISPOR ANNOUNCES ESTABLISHMENT OF NEW INITIATIVE ON VALUE ASSESSMENT FRAMEWORKS

ISPOR ANNOUNCES ESTABLISHMENT OF NEW INITIATIVE ON VALUE ASSESSMENT FRAMEWORKS

Posted on May 5, 2016

ANNOUNCEMENT

Princeton, NJ—May 5, 2016—The International Society for Pharmacoeconomics and Outcomes Research (ISPOR) announced today that it is embarking on the planning phase of a new *Initiative on Value Assessment Frameworks*.

search here ... Go

Archives

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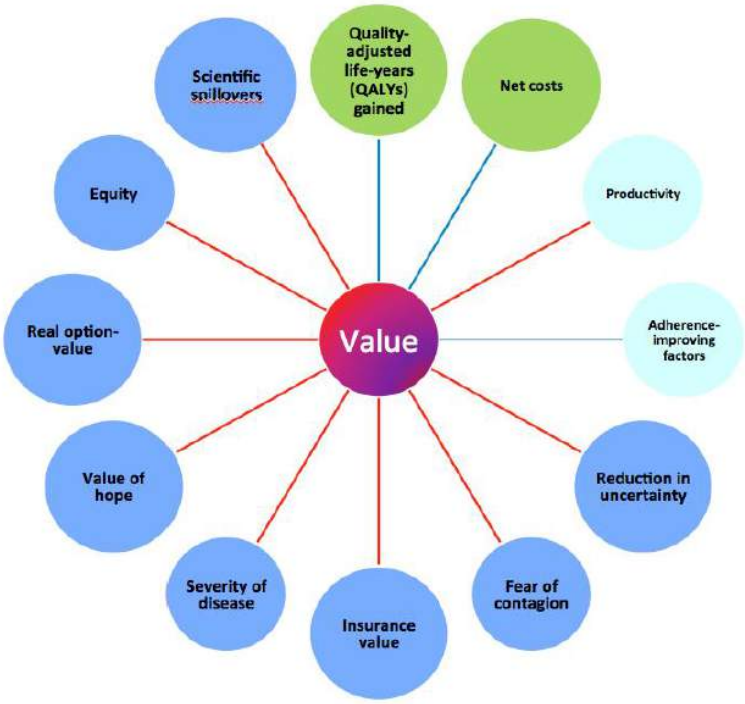
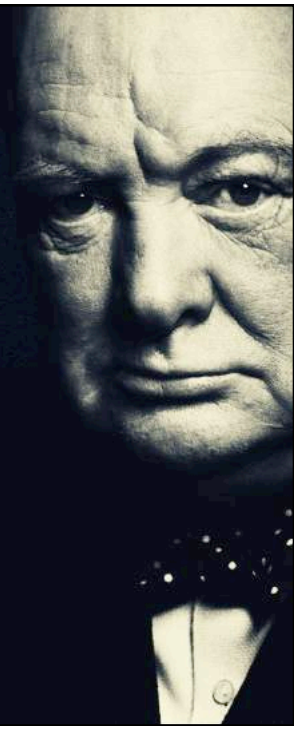
  - [+] March (6)
  - [+] February (4)
  - [+] January (2)
- [+] 2016 (75)



Many forms of Government have been tried, and will be tried in this world of sin and woe.

No one pretends that democracy is perfect or all-wise.

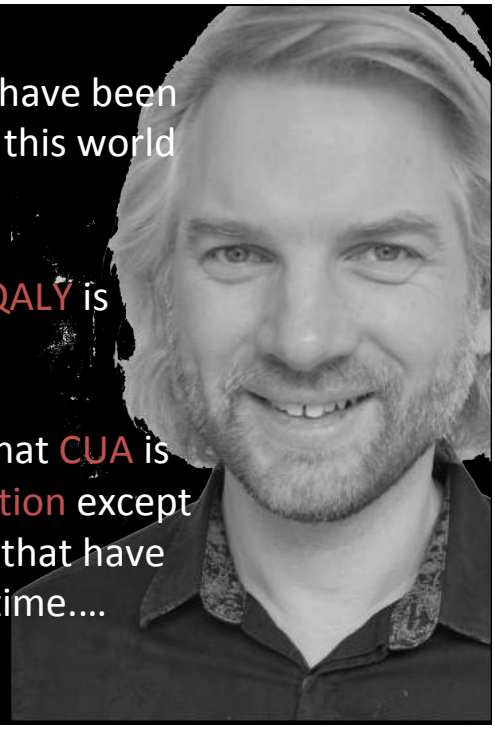
Indeed it has been said that democracy is the worst form of Government except for all those other forms that have been tried from time to time….



Many forms of outcome have been tried, and will be tried in this world of sin and woe.

No one pretends that a QALY is perfect or all-wise.

Indeed it has been said that CUA is the worst form of evaluation except for all those other forms that have been tried from time to time….



Focus groups on value at MSKCC

Ranking	Patients	Physicians	Nurses
1	Overall Efficacy	Efficacy	Efficacy
2	Side effects	Financial toxicity	Quality of Life
3	Long-term sequelae	Toxicity	Side-effects
4	How well established the treatment is	Functional outcomes	Administration
5	Reputation of the oncologist, cancer center, group	Inconvenience to patients/ caregivers and family	Communication
6	Alternative options	Societal costs and consequences	Innovation

University of Glasgow | Institute of Health & Wellbeing — HeHTA — Memorial Sloan Kettering Cancer Center

Identifying attributes of cancer treatments: What do stakeholders consider important?

Baba C., Briggs A[1], McIntosh E[1], Lipitz-Snyderman A[2], Kaltenboeck A[2], Bach PB[2]
[1]University of Glasgow, Glasgow, United Kingdom, [2]Memorial Sloan Kettering Cancer Center, New York, NY, USA

Background

Ever rising oncology therapy costs and a focus on patient-centred care has created a call for adjustments to existing pharmaceutical development models to assessing the value of new treatment options (1). These new models have sought to move away from solely focusing on the clinical benefits to also include wider 'value' considerations (2,3). Value-based pricing (VBP) seeks to examine how to achieve a fairer provision of care, and the possibility of incorporating other non-health, contextual treatment features deliberated by payers and stakeholders. However, there is lack of consensus as to what criteria beyond health should be included to define a treatment's value and how these criteria may vary dependent on stakeholder perspective (4).

Study objective

To identify non-health attributes prioritised by oncology clinicians, nurses and patients, stakeholders to aide the development of a new conceptual value framework for oncology.

Methodology

A structured focus group process employing the nominal group technique (5) was used to identify the attributes prioritized by different stakeholder groups. The 4 staged process is outlined below:
1. Idea –participants given 10 minutes to silently list their prioritised cancer treatment attributes;
2. Round Robin Stage - participants state attributes whilst the facilitator writes them on a board. The facilitator then suggests phrases of treatment attributes considered in existing value frameworks not stated by the group for review and possible addition to their collective list;
3. Clarification – the group discusses each treatment attribute, and produces a definition for each;
4. Ranking – First, individual silent ranking of the treatment attributes without discussion in order of descending importance. Second, group voting of the top six most important attributes.

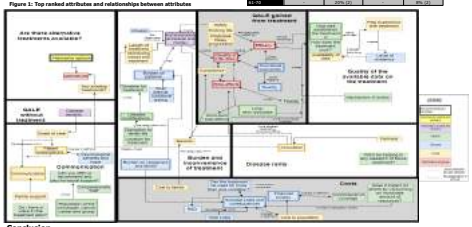
Participants

Recruited through Memorial Sloan Kettering Cancer Centre (MSKCC). Eligible participants were English speaking, 18 to 70 years old and either previous cancer patients or currently employed as oncology nurses and clinicians. Focus groups were divided by experience, creating three distinct stakeholder perspectives. Sessions were audio recorded ensuring that all discussions around the attributes could be transcribed. Transcriptions were anonymised to protect the identities of the respondents.

Table 2: Top ranked non-health attributes

Focus group	Activity	Ranking		
		1	2	3
Patients	Individual	How well established the treatment is	Reputation of the oncologist, cancer center, group	Lifestyle modifications
	Group	How well established the treatment is	Reputation of the oncologist, cancer center, group	Alternative treatment options
Clinicians	Individual	Burden and inconvenience to patients	Financial toxicity	Burden and inconvenience on family and caregivers
	Group	Financial toxicity	Burden and inconvenience to patients	Societal costs and consequences
Nurses	Individual	Communication	Patient expectations	Barriers
	Group	Communication	Innovation	Patient expectations

Figure 1: Top ranked attributes and relationships between attributes



Data analysis

Participants' individual rankings were aggregated to produce a list of each focus group's cumulative importance scores for each attribute. Additionally, a list of all attributes identified through group voting was collated. Qualitative analysis of the transcripts was undertaken using NVivo software package (6) to facilitate coding. Key terms used by participants in discussions of each attribute were coded according to the treatment attribute they were being used to describe. This identified themes and contextual considerations associated with the attributes and captured any interactions between attributes and where themes were prevalent across multiple attributes.

Results

Three focus groups were conducted in February 2017 in New York City. A total sample of 24 participants was used, including 8 previous cancer patients, 10 currently practicing oncology nurses and 6 clinicians working within oncology. The socio-demographic characteristics of the participants are shown in Table 1. A total of 30 attributes were identified by the participants. Across all 3 focus groups issues of clinical efficacy and toxicity were prioritised. Table 2 presents the top ranked non-health attributes per group from the 2 ranking exercises. For the patients group, the quality of evidence on the treatment, how the treatment was established in the clinical practice, and the reputation of the oncologist and whether there were other treatment options available were prioritised. Concerns about impact on daily lives were also highly ranked by the clinicians (burden and inconvenience attributes). Concerns of cost both to society and to the patient was also prioritised (financial toxicity, barriers, societal costs and consequences). Lastly, in addition to reputation, the focus groups highlighted the importance of communication and managing patient expectations about their disease and treatment. Following identification of the top-ranked attributes, the research team grouped the attributes and their links to other (lesser prioritised) attributes into eight categories representing the top-ranked attribute considerations across the focus groups. These are shown in Figure 1. Figure 1 also shows the health-related attributes which were reclassified into Quality Adjusted Life Expectancy (QALE).

Table 1: Socio-demographic characteristics

Demographic	Patients	Nurses	Physicians	Total
Male	37% (3)	10% (1)	33% (2)	25% (6)
Female	63% (5)	90% (9)	67% (4)	75% (18)
18-30	12.5% (1)		17% (1)	8% (2)
31-40	25% (2)	10% (1)	83% (5)	42% (10)
41-50	50% (4)	10% (1)		21% (5)
51-60	12.5% (1)	40% (4)		21% (5)
61-70		40% (4)		8% (2)

Conclusion

The study illustrates how priorities when valuing oncology treatments differ by stakeholder group. Long-term adverse effects (sequelae), alternative treatment options, quality of evidence, how well established the treatment is and reputation of the treating oncologist/centre were prioritized by patients, whilst nurses prioritised mode of administration, quality of life, communication and treatment innovation. Clinicians focused on the burden and inconvenience of treatments, functional outcomes, financial toxicity to patients, and the societal costs and consequences from the treatment. This study identified a set of attributes and their inter-relationships to be taken forward for valuation within the next phase of the research, a discrete choice experiment survey.

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[3]Linley WG, Hughes DA. Societal views on NICE, cancer drugs fund and value-based pricing criteria for prioritising medicines: a cross-sectional survey of 4,548 adults in Great Britain. Health Economics. 2013;22(8):948-64.
[4]Drummond M, Towse A, Nicod E, Annemans L, Garrison L, Jonsson B, et al. Emerging issues in assessing the value of medicines to society. Value in Health. 2014;17(1):63-70.
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But will they trade health? Developing an economic value framework for oncology

Briggs AH[1,2], Baba C[1], Lipitz-Snyderman A[2], Kaltenboeck A[2], McIntosh E[1] Bach PB[2]
[1]Health Economics & Health Technology Assessment, University of Glasgow, Glasgow, United Kingdom, [2]Center for Health Policy & Outcomes, Memorial Sloan Kettering Cancer Center, New York, NY, USA

Background

Value frameworks in health care are proliferating often with very little input from economists. In particular, a number of oncology value frameworks have emerged, perhaps due to the greater costs of novel therapies in the cancer space. But none of these value frameworks include weights that economists would recognise as legitimate values. If attributes beyond health are truly valued then it is reasonable to suppose that health would be traded for other attributes of value. One test of value would be to see if stakeholders would trade a health attribute for non-health attributes.

Study objective

Building on previous qualitative work that identified health and non-health attributes of cancer treatment, the aim of this study was to design and pilot test a discrete choice experiment (DCE) to identify trade-offs between identified attributes.

Methodology

Previously reported qualitative focus group work with cancer patients, oncology clinicians and oncology nurses identified the following as important attributes alongside the traditional health gain associated with treatment:

- treatment convenience;
- existence of treatment alternatives;
- disease rarity;
- quality of evidence;
- prognosis without treatment.

These results informed the design and undertaking of a DCE piloted in June 2017 with a convenience sample (n=45) where subjects where asked to choose between covering two alternative treatments in a new health plan. Quality-adjusted life expectancy (QALE) is used as a payment vehicle and the pilot tested whether respondents would be willing to give up health gains associated with treatment for the other identified attributes. An example DCE question from the designed survey is presented in **Box 1** below.

Box 1: example of DCE Question

In this questionnaire we want you to imagine that you have been tasked with advising a new government committee. This committee is in the process of designing a new, experimental health insurance plan. The committee have asked you to review a number of treatment options for different diseases and to choose which you would suggest for them to fund in this new health plan. Please assume that everything else about the treatment options is the same, including the **COST**.

Which of these treatments would you choose?

	Treatment A	Treatment B	
Inconvenience of treatment to patients and family	**SLIGHTLY INCONVENIENT** : Treatment is locally available, requires a low frequency of hospital visits and will not require patients to stop working	**SLIGHTLY INCONVENIENT** : Treatment is locally available, requires a low frequency of hospital visits and will not require patients to stop working	
Are there alternative treatments available?	**NO**: This treatment does not work there are no future care options and is the end of the line for the patient	**YES**: This treatment does not work there are other alternative care options available to the patient	**Neither**
Number of people affected with disease in US population per year	**1 in 500**	**1 in 10,000**	
Quality of available evidence on treatment	**LOW:** little data available from poorly-designed studies	**LOW:** little data available from poorly-designed studies	
Quality-Adjusted Life Expectancy gained WITH treatment	**24 MONTHS**	**12 MONTHS**	
Quality-Adjusted Life Expectancy gained WITH treatment	**1 MONTH**	**3 MONTHS**	
Which treatment would you prioritise?	**0**	**0**	**0**

Results

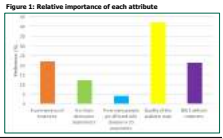
Mixed logit regression (**Table 1**) identified statistically significant preferences for less inconvenient treatments, treatments with no alternatives, higher evidence quality, helping those with shorter prognosis and provide more QALE gains. Only the number of people affected (rarity of disease) was insignificant.

Table 1: Mixed Logit Regression

Attribute	Coef.	P-value	95% CI
Inconvenience of treatment			
Very			
Slightly	0.89 (0.22)*	0.00	0.46, 1.31
Alternative treatments available			
No			
Yes	-0.5 (0.22)*	0.02	-0.93, -0.07
Number of people affected by disease/year			
1 in 10,000			
1 in 500	-0.18 (0.21)	0.39	-0.61, 0.24
1 in 50	0.05 (0.27)*	0.83	-0.4, 0.49
Quality of evidence			
Low			
Average	0.15 (0.21)	0.48	-0.26, 0.56
High	1.64 (0.34)*	0.00	0.98, 2.30
QALE without treatment			
3 months			
12 months	-0.09 (0.21)	0.68	-0.5, 0.33
24 months	-0.9 (0.31)*	0.00	-1.51, -0.28
QALE gained with treatment			
1 MONTH	0.65 (0.11)*	0.00	0.43, 0.86

In addition, the relative importance of each attribute was calculated and the results shown in Figure 1. At this stage of testing, and given the nature of using a convenience sample, the focus is placed on whether the results are significant and the relative value rather than attributing meaning to the magnitude of results. Nevertheless, the emphasis placed on data quality may have represented the bias of a health care based convenience sample.

Figure 1: Relative importance of each attribute



Conclusion

The pilot phase of this ongoing study demonstrates that whilst the health-gains of cancer therapies are predominantly prioritised, other attributes are important and participants were prepared to trade health gains. The pilot results will be used to inform the design of a larger study to identify the relative weight for the creation of a value framework in oncology with economically robust weights, and to explore the differences in weights expressed by different stakeholders.

Reflections on MCDA for HTA

- MCDA originally designed to help committees make decisions
- While extension to HTA seems intuitive, the 'devil is in the detail'
- Application to HTA requires careful consideration of:
  - Independence of criteria
  - Scoring that involves 'sacrifice'
- Economists use a particular form of MCDA

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Treacle and Smallpox: Two Tests for Multicriteria Decision Analysis Models in Health Technology Assessment

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ABSTRACT

Multicriteria decision analysis (MCDA) is rightly receiving increasing attention in health technology assessment. Nevertheless, a distinguishing feature of the health domain is that technologies must actually improve health, and good performance on other criteria cannot compensate for failure to do so. We argue for two reasonable tests for MCDA models: the treacle test (can a winning intervention be incompletely ineffective?) and the smallpox test (can a winning intervention be for a disease that no one suffers from?). We explore why models might fail such tests (as the models of some existing published studies would do) and offer some suggestions as to how practice should be improved.

Keywords: decision analysis, health technology assessment, multicriteria decision analysis, preferential independence.

Principles for Value Frameworks

- Should be generic not disease specific
- Should measure health in QALY terms
- Value beyond health attributes should be demonstrated by preparedness to sacrifice health
- Should pass the 'Treacle' and 'Smallpox' tests

Where next for Value Frameworks?

- US Panel and ISPOR/ASHEcon White Paper are aligned
- Value is in the Health Economists purview
- Could a broader concept of value re-ignite the welfarist / extra-welfarist debate?
- Many emerging value frameworks do not involve economists and do not conform to our notion of value
- We need to engage with 'value-quackery' in the medical field not the health economics literature