

# From Priceless Health to Impactful Economic Evaluation

Negotiating values friction for better decision-making

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# Outline of presentation

- Background to the plenary
- Background to economic evaluation in health care
- Things we have done well
- Things we have done not so well
- Conclusions

# Background to the plenary

- Economic evaluation has been impactful in the healthcare field, being a prominent component of decisions on the adoption of new health technologies in many countries
- In many ways this is surprising, given the independence of the medical profession and the more general view that one shouldn't put a price on health and human life
- Therefore, it might be interesting to explore the lessons learned from the the successes and failures of economic evaluation in healthcare

# The Donabedian Model for Evaluating the Quality of Healthcare

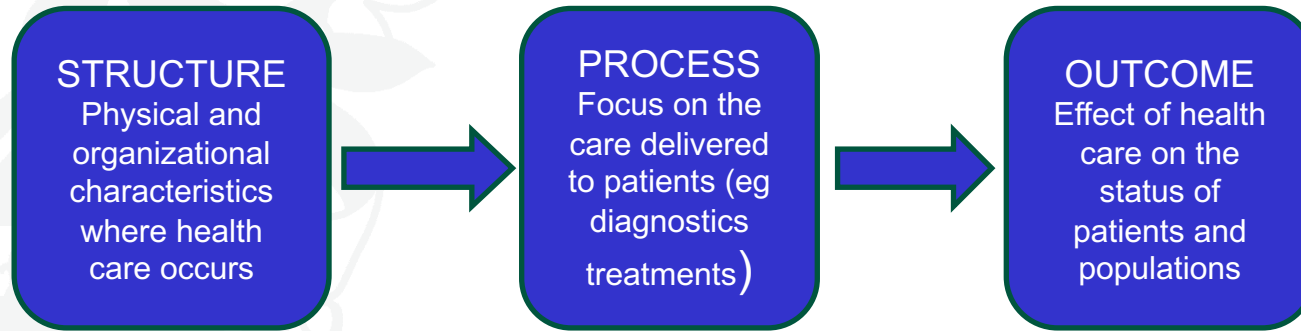


Donabedian, A (2005) Evaluating the Quality of Medical Care, *The Milbank Quarterly*, 83(4):691-729.  
(Originally developed in 1966)

# Background to economic evaluation in healthcare

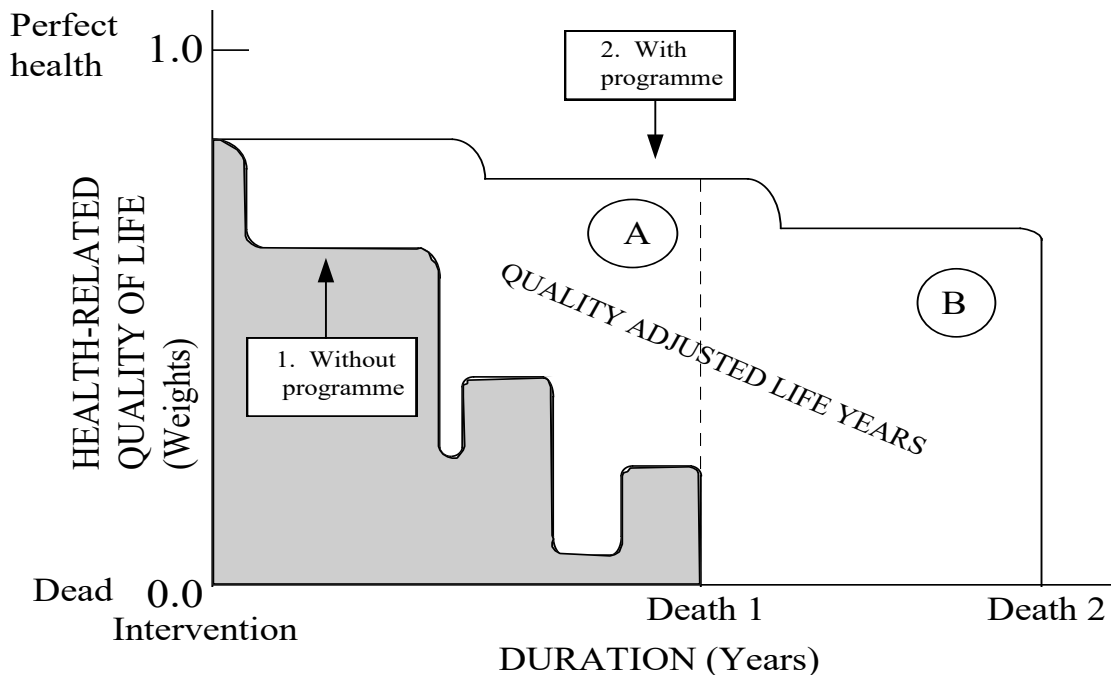
- Economic evaluations compare the costs and consequences of alternative courses of action
- There are several types of study (eg cost-effectiveness analysis, cost-benefit analysis), which differ in how the consequences are measured and valued)
- Monetary valuation of health outcomes has been largely avoided, and many studies used a generic measure of health improvement, such as the quality-adjusted life-year (QALY)
- The main application of economic evaluation in health care has been as part of health technology assessments (HTAs)

# The Donabedian Model for Evaluating the Quality of Healthcare



Donabedian, A (2005) Evaluating the Quality of Medical Care, *The Milbank Quarterly*, 83(4):691-729.  
(Originally developed in 1966)

# QALYs gained from a health intervention



# Overview of the HTA Process

- Identifying topics for assessment
- Specifying the decision problem
- Searching for evidence
- **Systematic review of the clinical evidence**
- **Economic evaluation**
- Assessing social, legal and ethical implications
- Formulating recommendations and implementation of policies
- Monitoring impact

## Note:

- The steps may not be tackled in this exact order; HTA is sometimes iterative
- The quantitative elements of HTA is shown in red. These steps are often tackled in one, integrated, analysis



# Brief history of economic evaluation in healthcare

- 1968 First cost-effectiveness studies by Klarman (renal dialysis) in the US and Pole (mass miniature radiography) in the UK
- 1976 First use of the term 'quality-adjusted life-years' by Harvard researchers
- 1990 Development of disability-adjusted life-year estimates by the World Bank and WHO, as part of the Bank's report on health
- 1991 Proposals by the Australian government to use cost-effectiveness criteria in the listing of drugs on the national formulary (Pharmaceutical Benefits Scheme) (implemented in 1993)

# Brief history of economic evaluation in healthcare (cont.)

- 1992 Launch of the journal, *Pharmacoeconomics*
- International Society for Pharmacoeconomics and Outcomes Research (ISPOR) founded, growing to 20,000 members in 100+ countries by 2019
- 1998 Launch of ISPOR's journal, *Value in Health*
- 1999 National Institute for Clinical Excellence (NICE) established in the UK, offering guidance to the NHS on the adoption of new health technologies
- 2014 The Institute for Clinical and Economic Review (ICER) began producing economic evaluations of new drugs to assist US private health plans in formulary decision-making
- 2023 The Inflation Reduction Act in the US will allow Medicare to consider economic criteria in its price negotiations with pharma companies on financially significant drugs after 7 years on the market



***“The drug itself has no side effects -  
but the number of health economists needed to  
prove its value may cause dizziness and nausea”***

# Things we have done well

- Winning over the professionals
- Simplifying the message
- Connecting with the decision-making process
- Developing analytic standards

# Winning over the professionals

- Initially there was quite considerable hostility from the medical profession, as in many healthcare systems doctors' decisions were not questioned
- We made a distinction between treatment decisions for an individual patient, and planning decisions for a population or community of individuals
- We were helped considerably by the growth of the 'Evidence-Based Medicine' movement, whereby the medical profession was developing its own approaches to evaluation
- Over time, use of economic evaluation was stimulated by budgetary pressures in health care systems and the requirements of research granting organizations for including economic evaluations

# Simplifying the message

- Like most disciplines, economics has its own jargon that doesn't facilitate interaction with other professionals; although economists had to learn the medical jargon
- The central concept in economics is 'opportunity cost'; the *cost* of using a resource (eg an hour of a doctor's time) is *the benefits that it would have generated in its best alternative use*
- This was considered to be a bit esoteric, so someone came up with the slogan '*Think of the patient who isn't in the room*'
- Slogans can have a big impact eg '*Slip, Slop, Slap*', '*Hands, Face, Space*'

# Connecting with the decision-making process

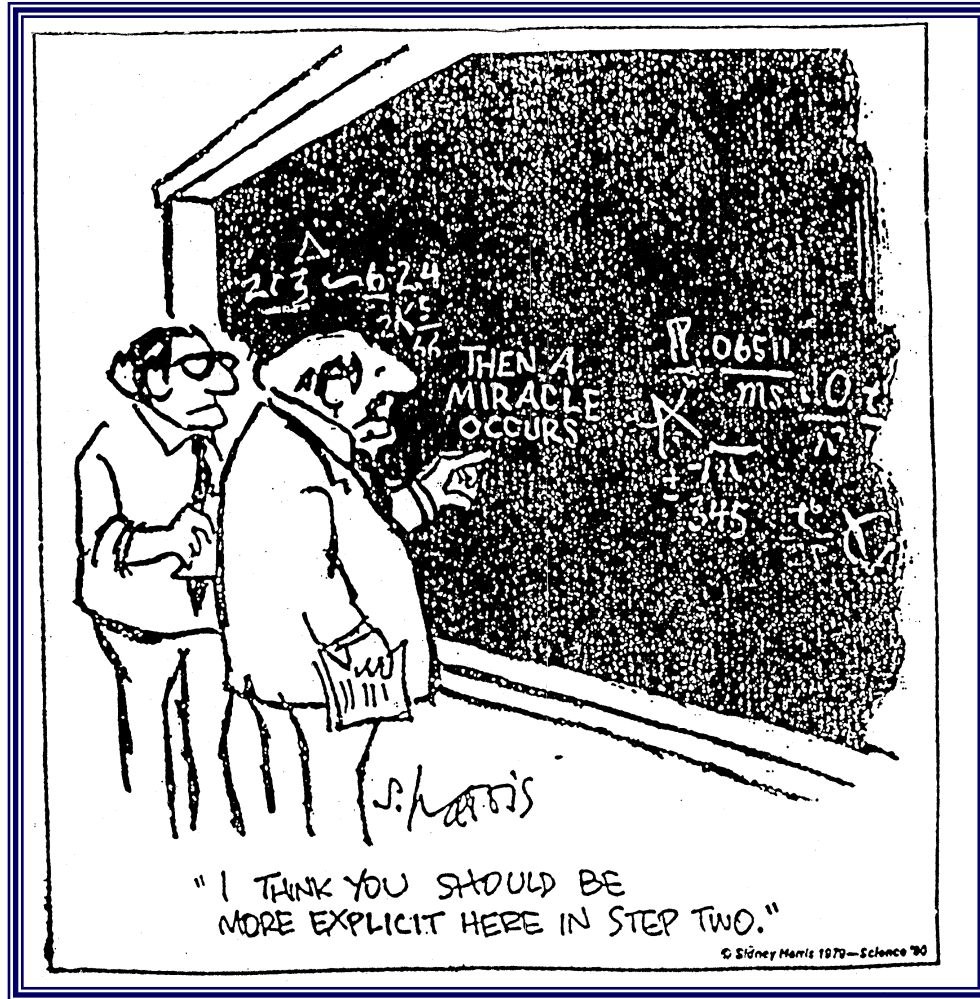
- Health economists tried hard to understand decision-makers' needs
- They have tried to offer solutions, rather than raise more questions
- They have built on existing decision-making structures, where these existed
  - eg In 1991 the PBAC already had a process for assessing the effectiveness of new drugs. It was relatively easy to add the economic evaluation to this process
- They have tried to formulate decision-makers' questions in ways that they could be answered by an evaluation
- Could these practices be a weakness, as well as a strength?

# Developing analytic standards

- For decision-makers, and other stakeholders, to trust economic evaluations, they have to trust the methods
- Evaluations also need to be reported in a transparent way
- Health economists have helped decision-makers to specify guidelines for the kind of evaluations they expect
- They have also developed reporting guidelines, consistent with those for reporting different types of clinical evaluations



The importance of clear reporting





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## ISPOR TASK FORCE REPORT

# Consolidated Health Economic Evaluation Reporting Standards (CHEERS)—Explanation and Elaboration: A Report of the ISPOR Health Economic Evaluation Publication Guidelines Good Reporting Practices Task Force

Don Husereau, BScPharm, MSc<sup>1,2,3,\*</sup>, Michael Drummond, PhD<sup>4</sup>, Stavros Petrou, MPhil, PhD<sup>5</sup>, Chris Carswell, MSc, MRPharmS<sup>6</sup>, David Moher, PhD<sup>7</sup>, Dan Greenberg, PhD<sup>8,9</sup>, Federico Augustovski, MD, MSc, PhD<sup>10,11</sup>, Andrew H. Briggs, MSc (York), MSc (Oxon), DPhil (Oxon)<sup>1,2</sup>, Josephine Mauskopf, PhD<sup>13</sup>, Elizabeth Loder, MD, MPH<sup>14,15</sup>, on behalf of the ISPOR Health Economic Evaluation Publication Guidelines - CHEERS Good Reporting Practices Task Force

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# Website Resources

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Improving health care decisions

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## CHEERS

ISPOR's CHEERS Report has been updated to reflect the most recent economic evaluation methods.

- About HEOR
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Consolidated Health Economic Evaluation Reporting Standards (CHEERS) 2022

CHEERS 2022 has been updated to reflect recent developments in economic evaluation methods and replaces the original 2013 CHEERS guidance for reporting health economics research. CHEERS 2022 is recognized by the EQUATOR Network as a reporting guideline for health research studies along with CONSORT, STROBE, and PRISMA. CHEERS 2022 has been endorsed and copublished by more than 15 journals (see "CHEERS is Endorsed by..." below).

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**Consolidated Health Economic Evaluation Reporting Standards (CHEERS) Statement**

Reporting guideline provided for? (i.e. exactly what the authors state in the paper)

Full bibliographic reference

Economic evaluations of health interventions

Husereau D, Drummond M, Petrou S, Carswell C, Moher D, Greenberg D, Augustovski F, Briggs AH, Mauskopf J, Loder E. Consolidated Health Economic Evaluation Reporting Standards (CHEERS) statement.

This guideline was published simultaneously in 10 journals. You can read the guideline in any of these journals using the links below.

Eur J Health Econ. 2013;14(3):367-372. PMID: 23526140  
 Value Health. 2013;16(2):e1-e5. PMID: 23538200  
 Clin Ther. 2013;35(4):356-363. PMID: 23537754  
 Cost Eff Resour Alloc. 2013;11(1):6. PMID: 23531194  
 BMC Med. 2013;11:90. PMID: 23531128  
 BMJ. 2013;346:f1049. PMID: 23529982  
 Pharmacoeconomics. 2013;31(5):381-387. PMID: 23529207  
 J Med Econ. 2013;16(6):713-719. PMID: 23521534  
 Int J Technol Assess Health Care. 2013;29(2):117-122. PMID: 23587340  
 BJOG. 2013;120(6):765-770. PMID: 23565948

**Reporting guidelines for main study types**

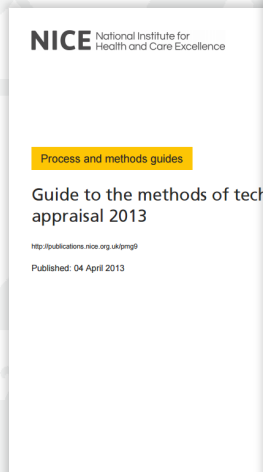
<b>Randomised trials</b>	CONSORT	Extensions
<b>Observational studies</b>	STROBE	Extensions
<b>Systematic reviews</b>	PRISMA	Extensions
<b>Study protocols</b>	SPIRIT	PRISMA-P
<b>Diagnostic/prognostic studies</b>	STARD	TRIPOD
<b>Case reports</b>	CARE	Extensions
<b>Clinical practice guidelines</b>	AGREE	RIGHT
<b>Qualitative research studies</b>	SRQR	COREQ
<b>Animal pre-clinical studies</b>	ARRIVE	
<b>Quality improvement studies</b>	SQUIRE	Extensions
<b>Economic evaluations</b>	CHEERS	

[equator-network.org/reporting-guidelines/cheers/](http://equator-network.org/reporting-guidelines/cheers/)

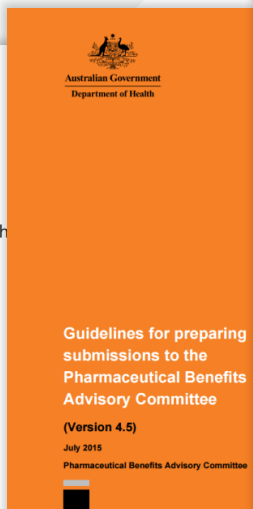
[www.ispor.org/cheers](http://www.ispor.org/cheers)

# Current economic evaluation guidelines outlined by HTA organizations

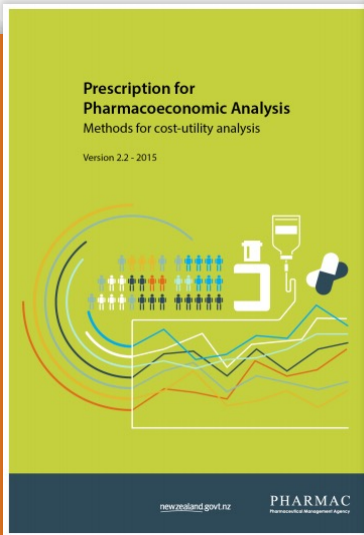
## England & Wales<sup>1</sup>



## Australia<sup>2</sup>



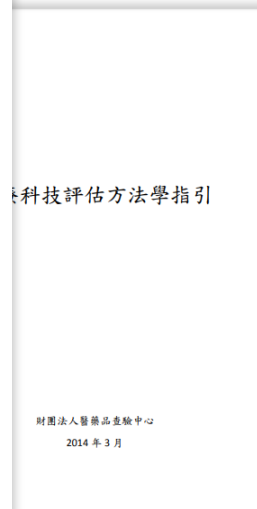
## New Zealand<sup>3</sup>



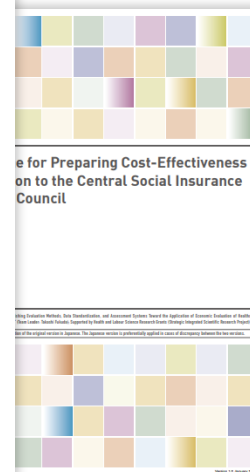
## South Korea<sup>4</sup>



## Taiwan<sup>5</sup>



## Japan<sup>6</sup>



- 1. NICE (2013). Guide to the methods of technology appraisal 2013. Available at: <https://www.nice.org.uk/process/pmg9/resources/guide-to-the-methods-of-technology-appraisal-2013-pdf-2007975843781>. Last accessed September 2020; 2. Australian Department of Health (2016). Guidelines for preparing a submission to the Pharmaceutical Benefits Advisory Committee. Available at: <https://pbac.pbs.gov.au/content/information/files/pbac-guidelines-version-5.pdf>. Last accessed September 2020; 3. Pharmac (2015). Prescription for Pharmacoeconomic Analysis: Methods for cost utility analysis. Available at: <https://www.pharmac.govt.nz/assets/pfpa-2-2.pdf>. Last accessed September 2020; 4. HIRA (2006). Pharmaceutical Economic Evaluation Guidelines [Korean]. Available at: [https://tools.ispor.org/PEGuidelines/source/Korean\\_PE\\_Guidelines\\_Korean\\_Version.pdf](https://tools.ispor.org/PEGuidelines/source/Korean_PE_Guidelines_Korean_Version.pdf). Last accessed September 2020; 5. TASPOR (2014). Guidelines of Methodological Standards for Pharmacoeconomic Evaluations [Mandarin]. Available at: [https://tools.ispor.org/PEGuidelines/source/HTA\\_guidelines\\_Taiwan.pdf](https://tools.ispor.org/PEGuidelines/source/HTA_guidelines_Taiwan.pdf). Last accessed September 2020; 6. Fukuda T, et al. (2017). Guideline for preparing cost-effectiveness evaluation to the central social insurance medical council. Available at [https://tools.ispor.org/PEGuidelines/source/Japanese\\_PE\\_Guideline.pdf](https://tools.ispor.org/PEGuidelines/source/Japanese_PE_Guideline.pdf). Last accessed September 2020.

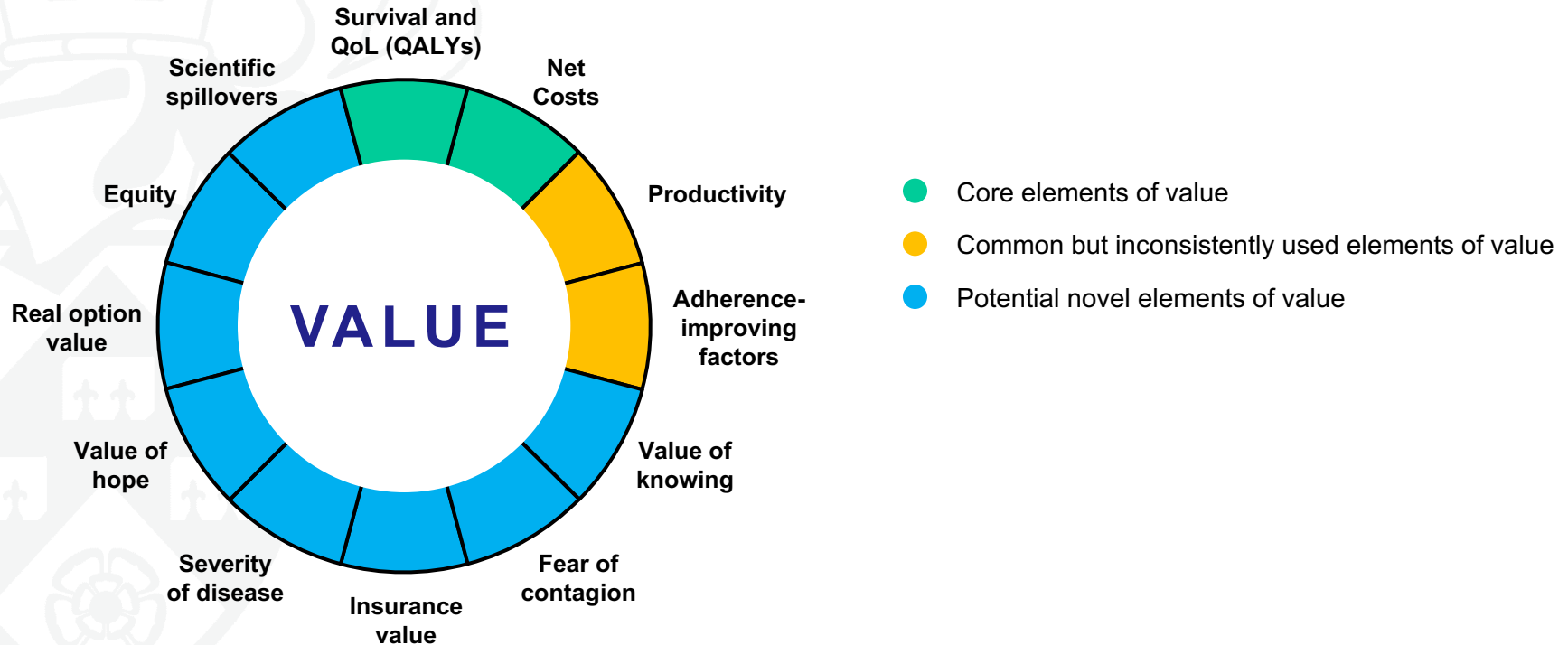
# Things we have not done so well

- Considering multi-faceted notions of value
- Dealing with the lack of controlled comparisons
- Tackling more complex choices
- Dealing with unquantifiable uncertainty

# Considering multi-faceted notions of value

- Evaluations in health care have focused mainly on *outcomes* in terms of improvements in length and quality of life
- These have been presented as multiple outcomes, or combined in a measure such as the QALY or DALY; monetary valuation has been largely ignored, for ethical reasons
- A single outcome measure makes it easier to specify a decision rule (eg a cost-per-QALY threshold for accepting a new health technology)
- However, this is probably an over-simplification of the value of improved health
- There is already a growing interest in distributional CEA, which can deal with the equity issues

# Elements of Value According to the ISPOR Task Force <sup>1</sup>



- Figure adapted from Lakdawalla DN, *et al. Value Health* 2018;21:131–139.  
1. Drummond M, *et al. Value Health*. 2019;22(6):661–668.

# Dealing with the lack of controlled comparisons

- The Evidence-Based Medicine movement has generated many controlled comparisons of alternative therapies (through randomized controlled clinical trials)
- However, such clinical evidence is not available for many health technologies, especially medical devices or treatments for rare diseases
- Therefore, health economists have had to rely on historical controls or statistical solutions to make comparisons (eg propensity scoring or multivariate regression)
- However, evaluations have still focused on outcome, rather than process or structure (Donabedian, 2005)

Donabedian A. Evaluating the quality of medical care. *Milbank Quarterly* 2005; 83(4): 691-729



# Tackling more complex choices

- Part of the success of economic evaluation in health care has been due to simplifying the decision problem
- The pandemic has reminded us of the interactions between health and the rest of the economy; is a health care perspective (for analyses) sustainable?
- In most settings, decision-makers are interested in evaluating population, or system level, interventions as well as individual treatments
- More attention needs to be paid to the organizational impacts of adopting new technologies

Walker, S., Fox, A., Altunkaya, A., Colbourn, T., **Drummond, M.F.**, Griffin, S., Nutacker, N., Reville, P., Sculpher, M. Programme evaluation of population and system level policies: Evidence for decision-making. *Medical Decision Making* 2021

<https://doi.org/10.1177/0272989X211016427>.

# Dealing with unquantifiable uncertainty

- Health economists have developed methodologies for dealing with quantifiable uncertainty (eg where the probability of the event is known or can be assigned)
- These methods include probabilistic sensitivity analysis and value of information analysis
- But on some occasions the uncertainty cannot be quantified<sup>1</sup> (eg the durability of the effect of a new gene therapy)

1. Donald Rumsfeld, US Secretary of Defense (2002) ' *There are known knowns....., known unknowns....., and unknown unknowns* '

# Conclusions

- Economic evaluation in health care has been surprisingly successful, partly because of advantages arising from the availability of clinical outcome data, and the favourable environment caused by concerns about the use of healthcare resources
- As more complex decision problems are tackled, economic evaluation in health care is likely to encounter many of the problems experienced by evaluation in other fields
- However, evaluation doesn't have to be perfect in order to be useful



Don't let the perfect be the enemy of the merely good

# Summary: Economic Evaluation in Health Care

- ***Successes***

- gaining the confidence of professional groups
- simplifying the message
- connecting with decision-making process
- developing analytic standards

- ***Limitations***

- relatively simple concept of outcome/value
- little consideration of uncontrolled or complex choices/options
- problems with dealing with some uncertainties