

# Breakout Session 3: 138

How Much Weight Should be Placed on Additional Value Elements in Health Technology Assessment?

# Why are we here today?

- ▶ Current HTA relies on regulatory approval evidence to inform comparative- and cost-effectiveness calculations
- ▶ Conventional CEAs useful starting point for value assessments but may lead to suboptimal resource allocations and distorted signals to innovators<sup>1</sup>
- ▶ Return on investment for evidence generation on additional value elements is unclear

<sup>1</sup> Neumann PJ, Willke RJ, Garrison Jr LP. A health economics approach to US value assessment frameworks-introduction: an ISPOR Special Task Force report [1]. Value Health. 2018;21(2):119–123.

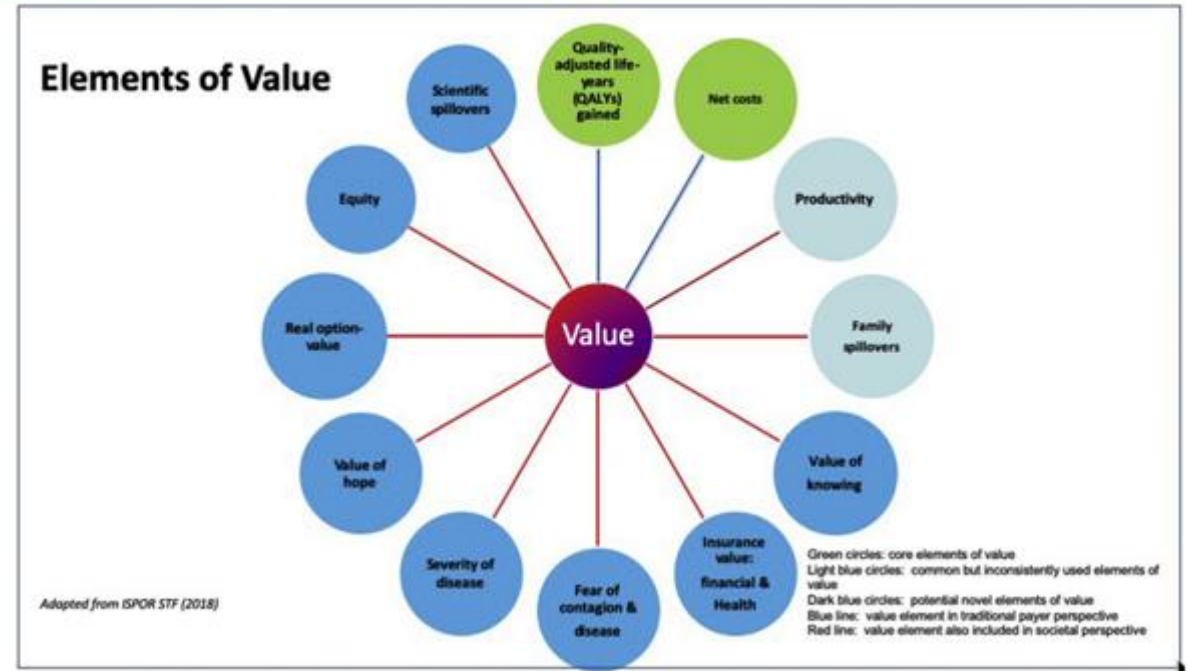
# Changing dynamic with modern technologies

- ▶ Rising prices for innovative technologies in the current system are *unsustainable* and not necessarily *justifiable*
  - Justifiable: did we correctly answer the value question?
  - Unsustainable: is current system of pricing and reimbursement sufficient to cover modern technologies?
- ▶ Evidence generation should be harmonized given the global nature of drug development
  - ***But what additional evidence should we consider and how much should we weigh that evidence versus conventional value?***

# Additional value elements<sup>2</sup>

- ▶ 2 core elements (QALYs and net costs)
- ▶ 2 common but inconsistently used elements (productivity and adherence)
- ▶ 8 potentially “novel” elements
- ▶ Additional impacts in Second Panel’s Impact Inventory Table

Figure 1. The ISPOR “Value Flower” 2018.



ISPOR STF indicates ISPOR Special Task Force; QALY, quality-adjusted life-year.

# Value interpretations... *It Depends*

- ▶ Other criteria influence US value interpretations<sup>3,4</sup>
  - Novel mechanism
  - Reduce caregiver burden
  - Lack of evidence
  - Uncertainty in long-term safety
- ▶ Despite different perspectives and decision contexts, significant overlap in valuing other novel criteria between payers and patients<sup>5</sup>

3 Neumann PJ et al. Should A Drug's Value Depend On The Disease Or Population It Treats? Insights From ICER's Value Assessments. Health Affairs Blog Nov 6, 2018;

4 Trenaman L, Pearson SD, Hoch JS. How Are Incremental Cost-Effectiveness, Contextual Considerations, and Other Benefits Viewed in Health Technology Assessment Recommendations in the United States? Value Health. 2020 May;23(5):576-584;

5 Jakab I et al. Criteria for Inclusion in a Multiple Criteria Decision Analysis: Comparing Patient and Payer Preferences for Additional Value Criteria. *Frontiers in Pharmacology* 2021 Jun 24; 12(69)



# Emerging supplements and alternatives

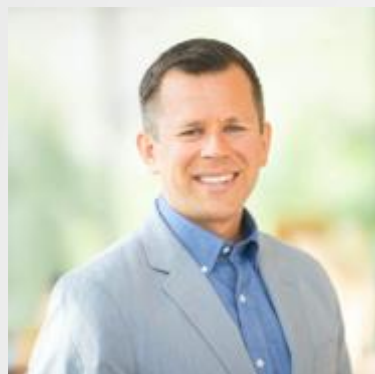
- ▶ Explicit inclusion in CEA calculations or thresholds:
  - Generalized risk adjusted CEA<sup>6</sup>
  - NICE's recent framework update includes severity modifier with potential to allow a higher threshold for treatments of rare diseases
- ▶ Expanded utility instruments covering more health domains
  - EQ-HWB<sup>7</sup>
- ▶ Multi-criteria decision analysis (MCDA) as supplemental tool<sup>8</sup>

6 Lakdawalla DN, Phelps CE. Health Technology Assessment With Diminishing Returns to Health: The Generalized Risk-Adjusted Cost-Effectiveness (GRACE) Approach. Value in Health. 2021 Feb;24(2):244-249

7 Monteiro et al. A Comparison of a Preliminary Version of the EQ-HWB Short and the 5-Level Version of the EQ-5D. Value in Health 2022.

8 McQueen RB, Slejko JF. Toward Modified Impact Inventory Tables to Facilitate Patient-Centered Value Assessment. Pharmacoeconomics. 2021 Apr;39(4):379-382

# Panelists



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# ISPOR 2022: NICE's value framework

Dr Sam Roberts  
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**NICE** National Institute for  
Health and Care Excellence





# What I will cover

- The NICE value framework and why we use it
- Structured decision making: what gets taken into account?
- Cases where other value elements have been considered by NICE

# NICE reference case for technology evaluation

- Reference case perspective reflects an objective of the English healthcare system to maximise *population health gains* from available resources (i.e. the NHS/PSS budget; not set by NICE)
- Non-health-outcomes and non-health areas of public spending are usually excluded

**NICE**

Element of health technology assessment	Reference case	Section providing details
Comparator(s)	As listed in the scope developed by NICE	2.2.4 to 2.2.6, 4.1.6, 4.1.14
Perspective on outcomes	All health effects, whether for patients or, when relevant, carers	4.1.7, 4.1.8
Perspective on costs	NHS and personal social services (PSS)	4.1.9 and 4.1.10
Types of economic evaluation	Cost-utility analysis with fully incremental analysis Cost-comparison analysis	4.1.11 to 4.1.14 4.1.18 to 4.1.22
Time horizon	Long enough to reflect all important differences in costs or outcomes between the technologies being compared	4.1.15 to 4.1.17
Synthesis of evidence on health effects	Based on systematic review	4.2
Measuring and valuing health effects*	Health effects should be expressed in quality-adjusted life years (QALYs). The EQ-5D is the preferred measure of health-related quality of life in adults	4.3.1
Source of data for measurement of health-related quality of life*	Reported directly by patients or carers, or both	4.3.3

# Why does NICE not use a societal perspective?

NICE

- Many technologies will have impacts outside the health system:
  - Enabling people to return to work or school
  - Impacts on use of other publicly-funded services
- But measuring all benefits and costs correctly may not be possible
- NHS budget is fixed, and transfers *between* sectors might not be feasible
- Ethical and legal constraints:
  - In 2014, proposals for incorporating 'wider societal benefits' and value-based assessment were considered by NICE but not implemented

# However...

In exceptional cases, the remit from the Department of Health and Social Care may permit consideration of non-health objectives

(to be presented separately from the reference case analysis)

# NICE evaluations that took non-health benefits into account

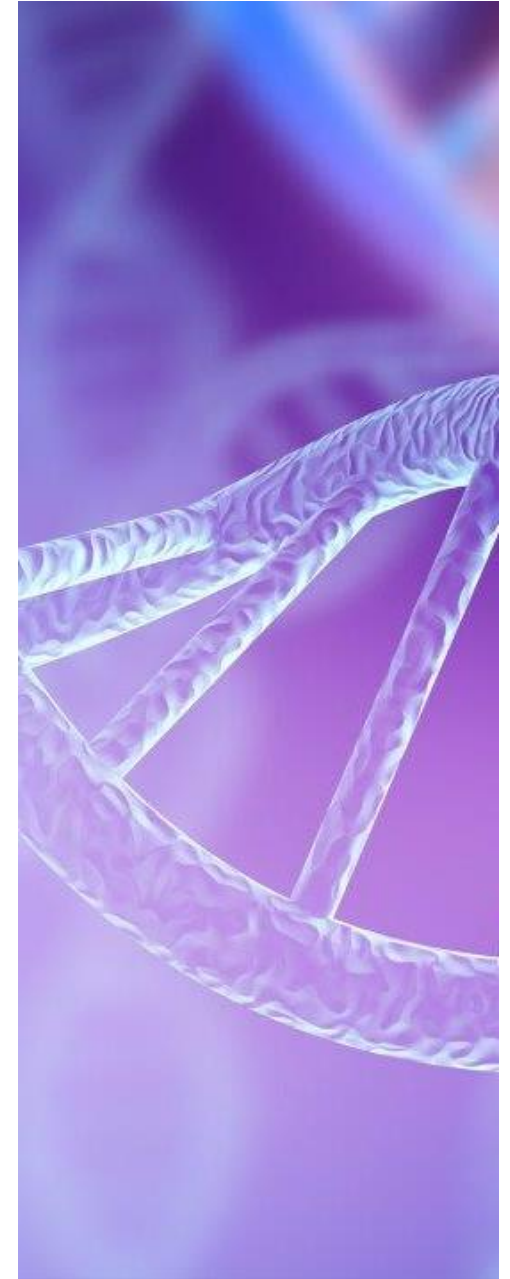
Nalmefene for reducing alcohol consumption in people with alcohol dependence (TA325; 2014)

- Costs and outcomes relevant to the criminal justice sector were taken into account

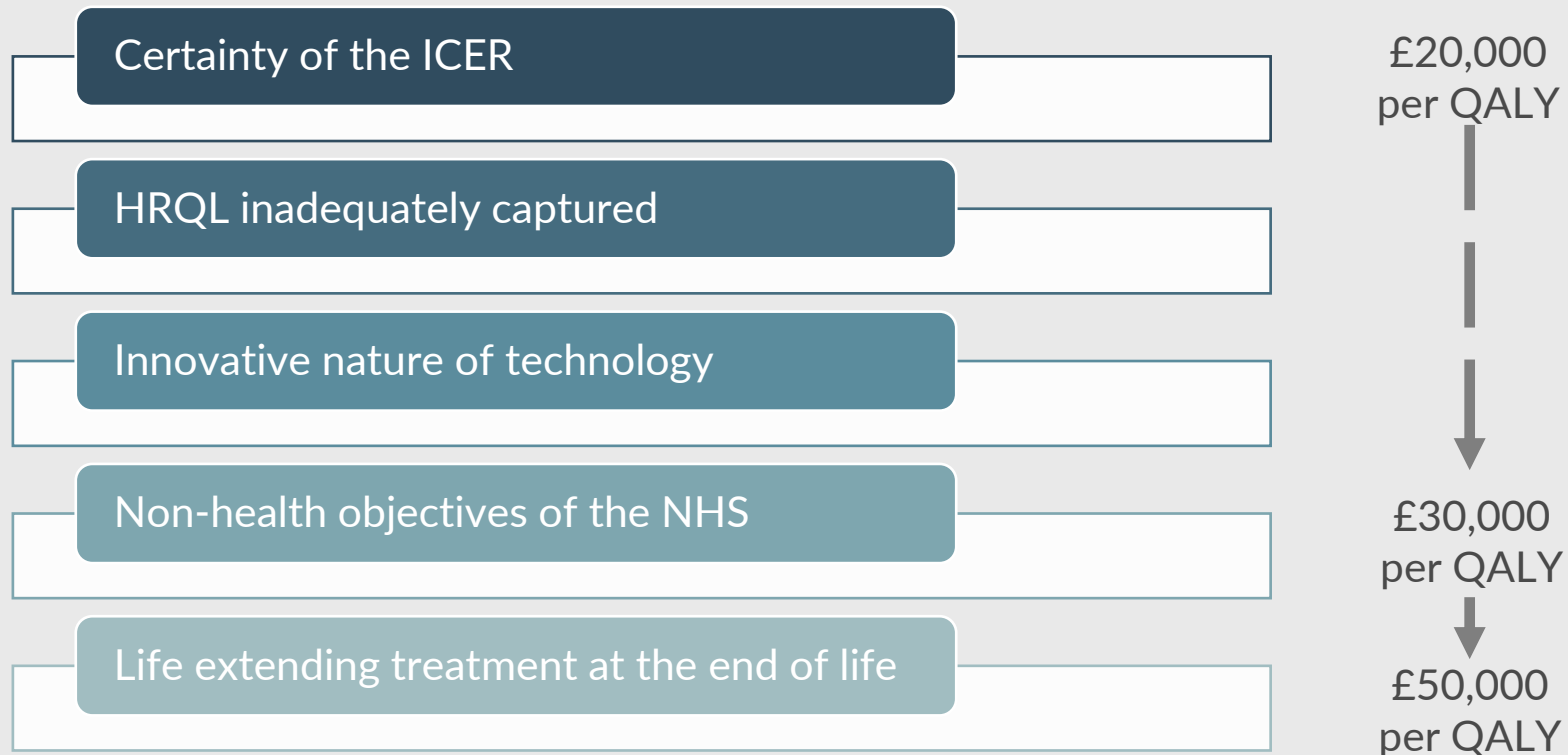
Cochlear implants for children and adults with severe to profound deafness (TA566; 2009, 2018)

- Benefits to education were taken into account

**NICE**



# Structured decision making 2013-2022



**NICE**

(For Highly Specialised Technologies, the threshold is £100,000 per QALY)



# NICE Principles

(previously  
known as social  
value  
judgements)

NICE

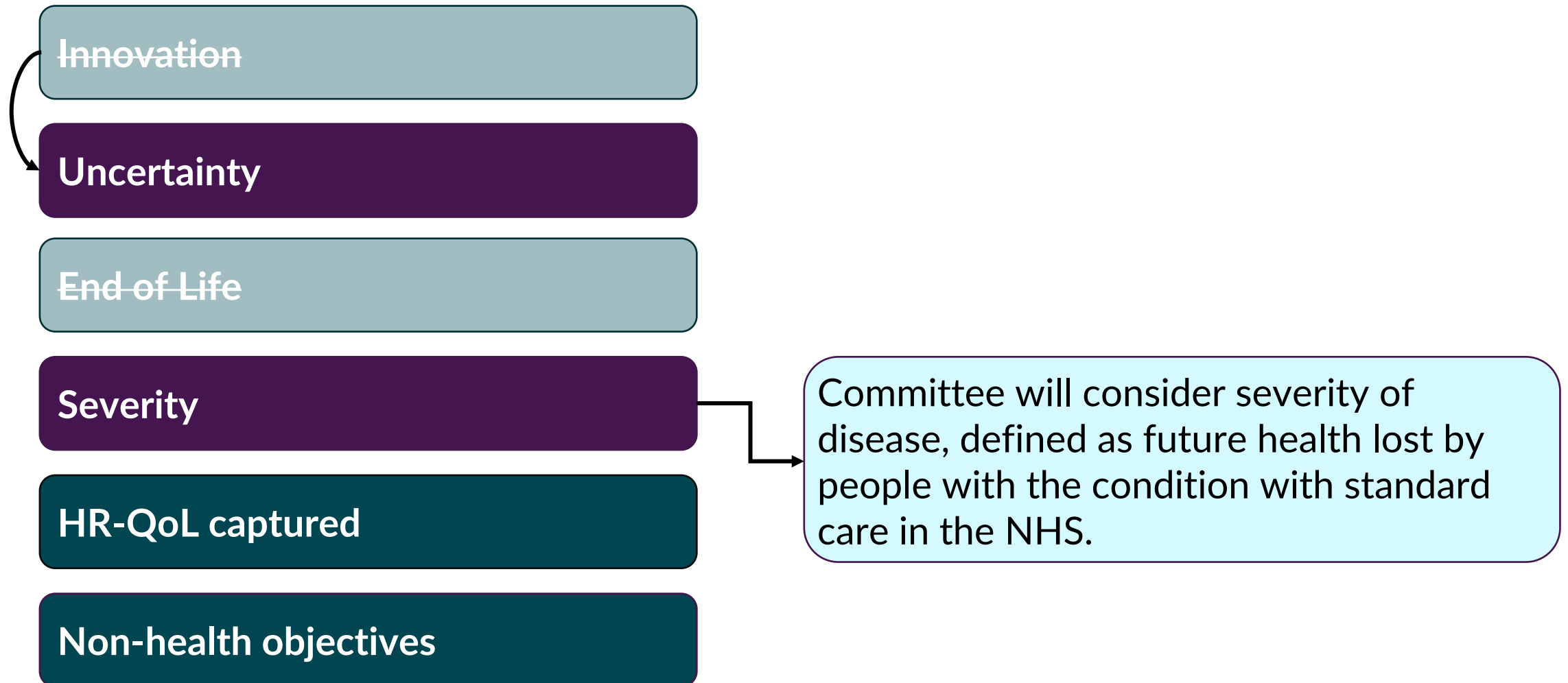


The 12 key principles that are universal to all of our guidance and standards. 2 principles specifically define what NICE guidance aims to achieve:

**Principle 8. Support innovation in the provision and organisation of health and social care services**

**Principle 9. Aim to reduce health inequalities**

# New methods and process manual (2022)



# Severity modifier

NICE

Evidence that society values benefits more for severe conditions

- Limited evidence for additional value placed on end of life conditions

## Severity modifier introduced

- Considerations for life-extending treatments at the **end of life** (the 'end of life criteria') **removed**

Severity considered as a **quantitative modifier** (for Technology Appraisals) that gives **additional weight** to health benefits in the most **severe conditions**

**Assessed using absolute and proportional QALY shortfall** (whichever is the highest)

Use a 2 step approach applying 1.2 and 1.7 weights to QALY gains (or losses)

QALY weight	<u>Proportional shortfall</u> or fraction of health lost	<u>Absolute shortfall</u> or total amount of health lost
1	Less than 0.85	Less than 12
x1.2	0.85 to 0.95	12 to 18
x1.7	At least 0.95	At least 18

## Two new drugs to fight superbugs available on NHS soon

11 Apr | [Health](#)

Two new medications which fight drug-resistant superbugs could soon become available to NHS patients in England.

Drugs' watchdog NICE says cefiderocol and ceftazidime with avibactam offer value for money and will save lives.

These 'last line of defence' drugs will tackle severe infections which cannot be cleared by other treatments.

Under the deal, a fixed annual fee will be paid to manufacturers to cover their development costs, rather than individual payments for each dose.

The subscription-style payment model is part of the government's strategy to incentivise companies to make essential drugs such as these, alongside the so-called blockbuster drugs which typically make huge profits for pharmaceutical giants.

# Value assessment of new antimicrobials

The evaluations considered the long-term value of the antimicrobials to the NHS in England including additional value elements

**NICE**

# Antimicrobials: wider elements of value

Element of value	What this means
Spectrum value	Benefits of replacing broad-spectrum antimicrobials with narrow-spectrum antimicrobials
Transmission value	Ability of the antimicrobials to reduce transmission of resistant pathogens over time
Enablement value	Enabling other medical procedures that require antimicrobial treatment (e.g. surgery, chemotherapy) in people with resistant pathogens
Diversity value	Having another antimicrobial available can reduce selection pressure on and resistance to other antimicrobials
Insurance value	potentially catastrophic situation where multi-drug resistance becomes so widespread that the antimicrobial is the only option across a large number of clinical scenarios.

# Conclusions

The value elements NICE considers in its evaluations are linked to the NHS context – a tax-funded healthcare system - and the remit NICE has

In its deliberations, NICE committees can place additional weight on certain value elements, such as disease severity, if appropriate

‘What is of value’ and ‘should that be funded from the NHS budget’ are separate questions and should be considered as such



Thank you.

**NICE** National Institute for  
Health and Care Excellence

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# How Much Weight Should be Placed on Additional Value Elements in Health Technology Assessment?

Jon Campbell, PhD

SVP for Health Economics



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# Disclosure

- ICER's funders are available online: <https://icer.org/who-we-are/independent-funding/>
- ICER's value assessments are free from financial conflicts of interest from life science companies and insurers
- Views are my own

# Gut sense

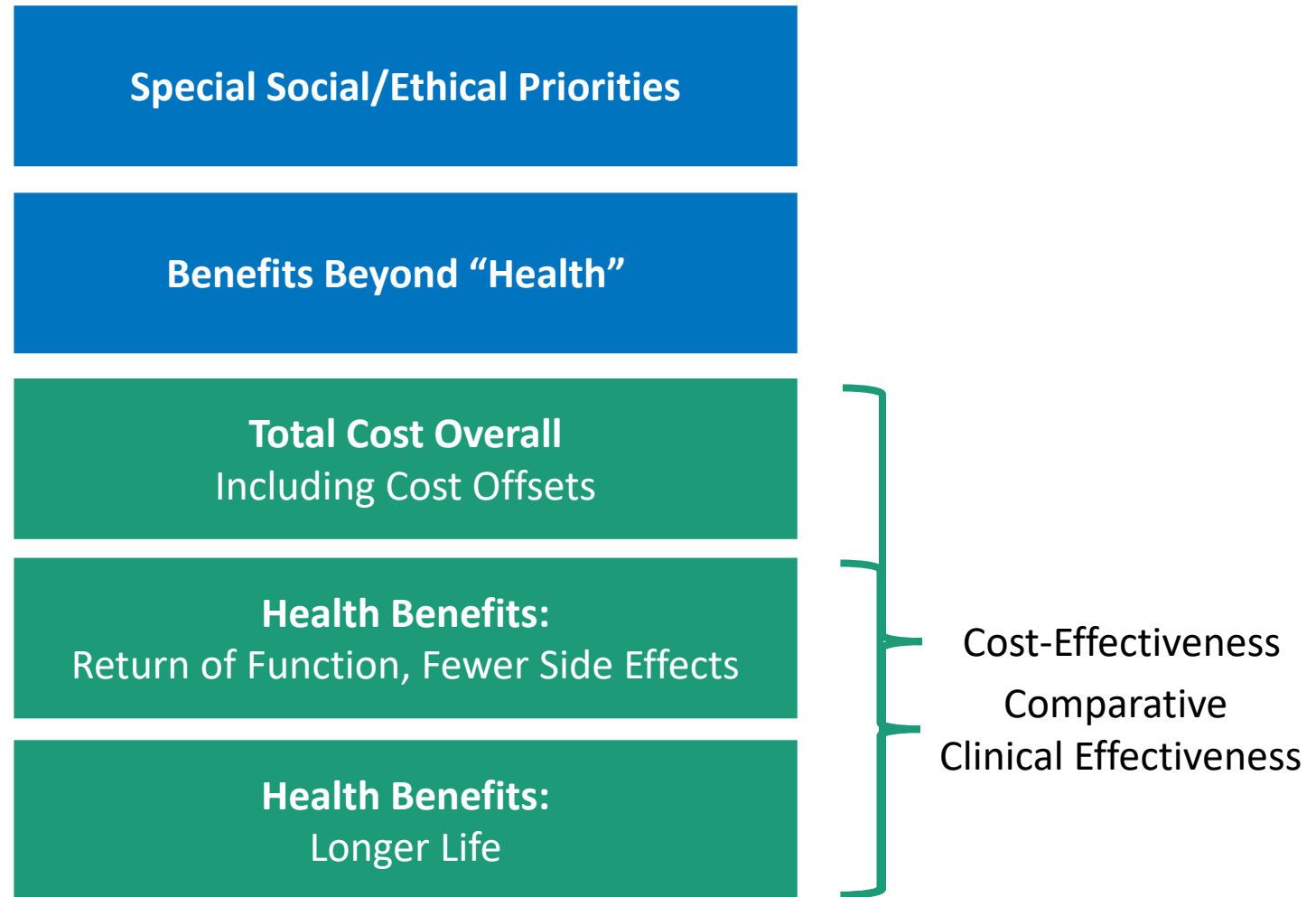


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# The ICER Value Framework: Purpose

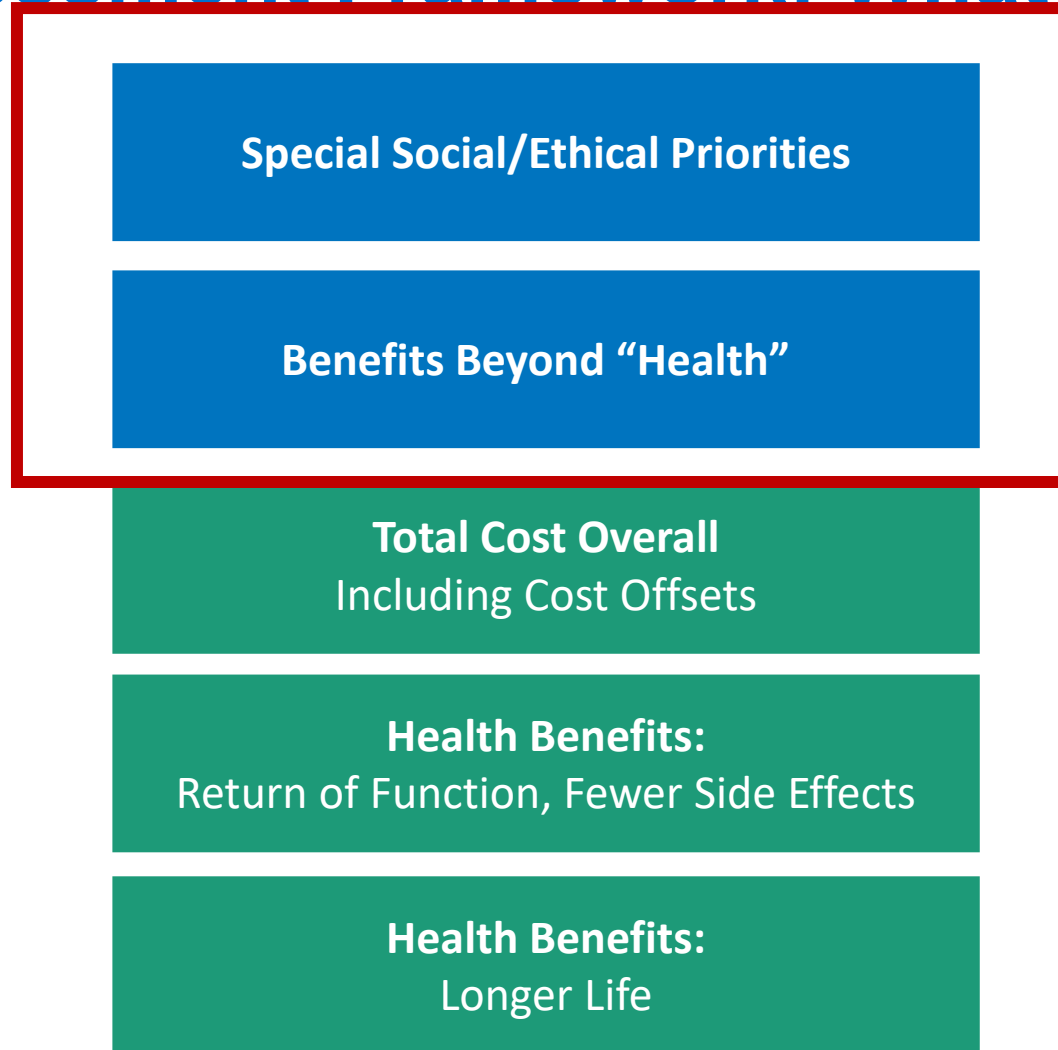
- Intended to make transparent how “value” is conceived of and evaluated in ICER reports
- Takes a “population” level perspective as opposed to trying to serve as a shared decision-making tool to be used by individual patients and their clinicians

# Value Assessment Framework: What is “Value”?





# Value Assessment Framework: What is “Value”?



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# Benefits Beyond Health and Social Priorities

- Severity of the condition
- Magnitude of lifetime burden of illness
- Broader effects on patients' ability to meet life goals
- Broader effects on caregivers' ability to meet life goals
- Ability to help society reduce health disparities

## Case Study (Benefits Beyond Health and Social Priorities)

When making judgments of overall long-term value for money, what is the relative priority that should be given to *any* effective treatment for \_\_\_\_\_, on the basis of the following contextual considerations:

Contextual Consideration	Very Low Priority	Low Priority	Average Priority	High Priority	Very High Priority
Acuity of need for treatment of individual patients based on the severity of the condition being treated	0	0	1	2	12
Magnitude of the lifetime impact on individual patients of the condition being treated	0	0	0	3	12

## Case Study (Benefits Beyond Health and Social Priorities)

What are the relative effects of \_\_\_\_\_ plus supportive care versus supportive care alone on the following outcomes that inform judgment of the overall long-term value for money of \_\_\_\_\_ ?

Potential Other Benefit or Disadvantage	Major Negative Effect	Minor Negative Effect	No Difference	Minor Positive Effect	Major Positive Effect
Patients' ability to achieve major life goals related to education, work, or family life	2	7	6	0	0
Caregivers' quality of life and/or ability to achieve major life goals related to education, work, or family life	3	7	4	0	0
Society's goal of reducing health inequities	9	4	1	1	0

**1. Consider Health Benefit  
Price Benchmark Range**

**2. Apply Considerations of  
Benefits Beyond Health  
and Special Priorities**

Price to reach  
\$100k/QALY or evLYG

Price to reach  
\$150k/QALY or evLYG

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# Future Directions

- ICER Value Assessment Update (2023)
  - Consider more numbers
    - Severity weighting
    - Modified societal perspective
    - Thresholds
  - Connect to the patient experience
  - Continue to encourage diverse discussion and deliberation

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# How Much Weight Should be Placed on Additional Value Elements in Health Technology Assessment?

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# How Much Weight Should be Placed on Additional Value Elements in Health Technology Assessment?

*- perspective of lower income countries -*

**Zoltán Kaló**

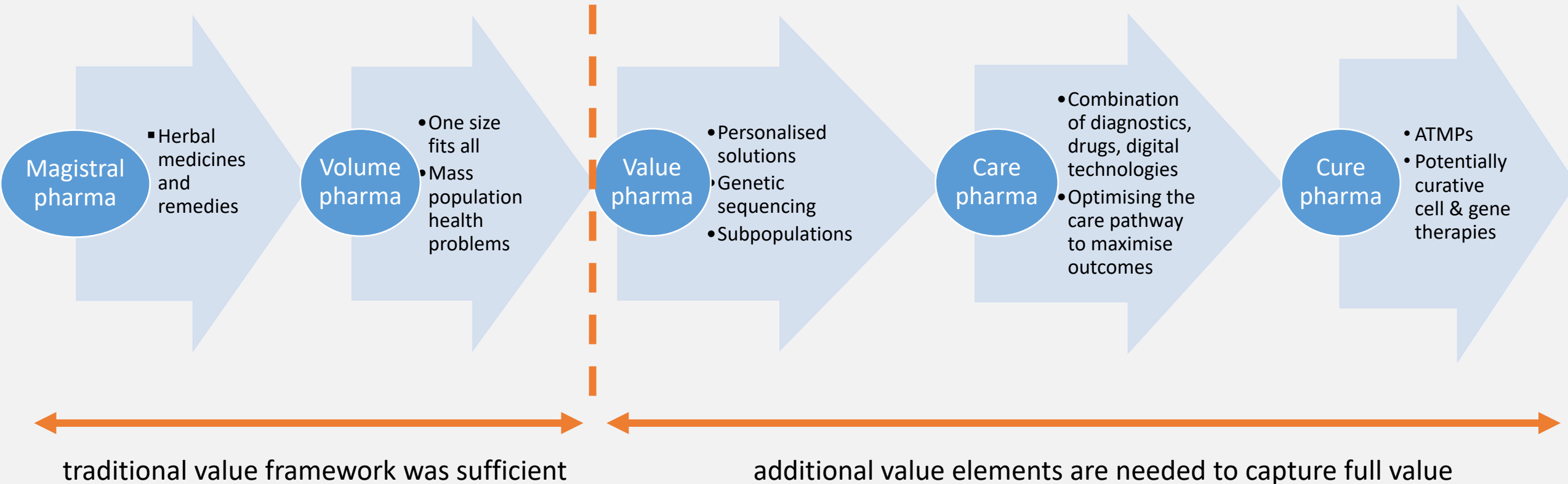
*Professor of Health Economics*

*1) Center for Health Technology Assessment Semmelweis University*

*2) Syreon Research Institute*



# R&D trends necessitate the extension of value frameworks



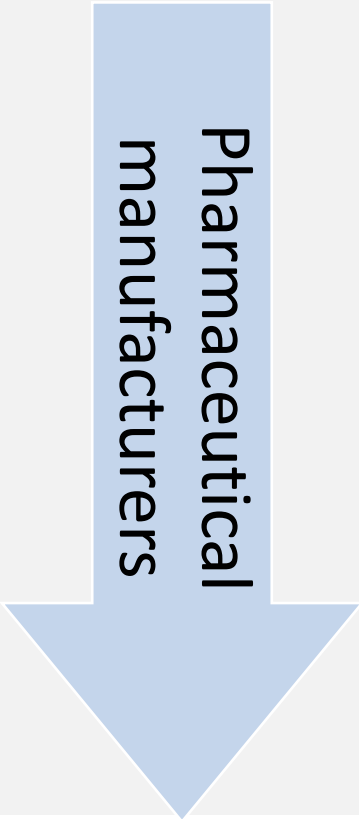
# Incentives of different stakeholders for additional value elements



Public policymakers

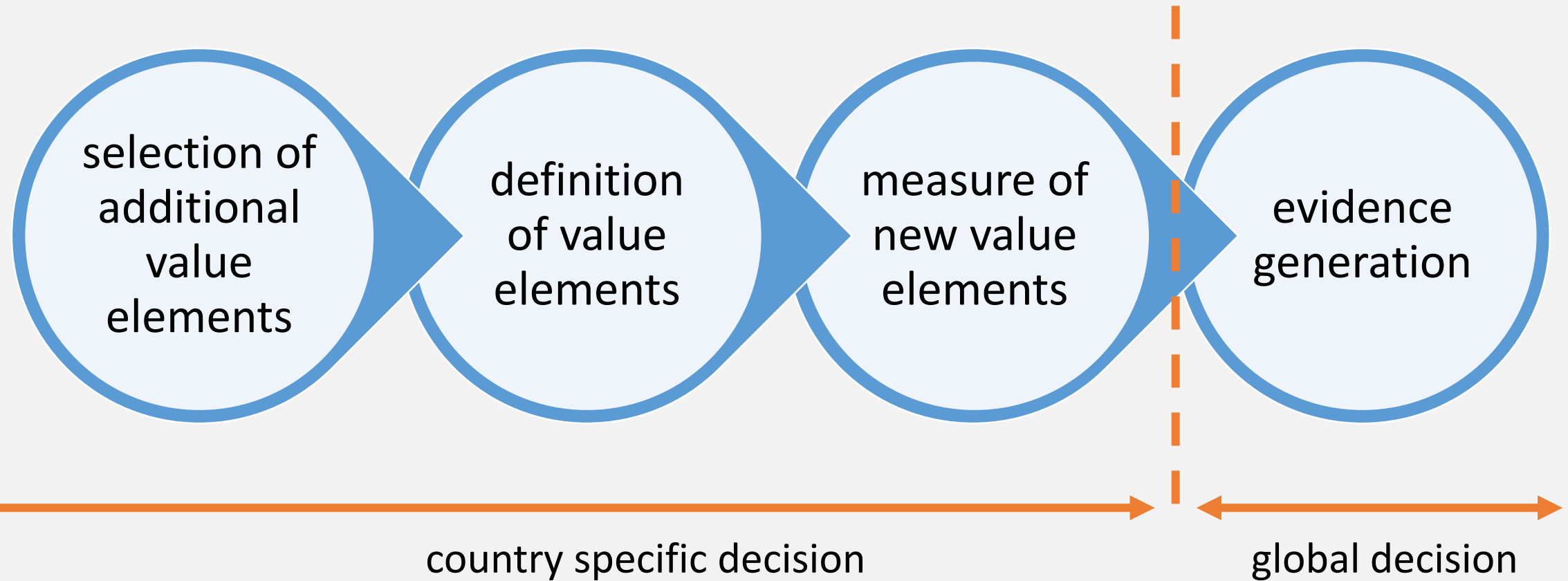
- Positive discrimination of patient centred and societally oriented health technologies
- Increased transparency and objectivity of policy decisions by explicit value judgement of previous implicit considerations

- Improved clarity on market access criteria
- Reduced impact of cost-effectiveness and budget impact analyses on policy decisions
- More emphasis on previously neglected value propositions



Pharmaceutical  
manufacturers

# Challenges of value maximisation of new health technologies based on extended value frameworks in a given market



# Complexity related to extended value frameworks in global markets

- Different countries may move into different directions with extending the value frameworks
- How can pharmaceutical industry manage diverse expectations in evidence requirements during the development phase?
- Without harmonization process of additional value criteria, the movement to extended value frameworks may even result in difficulties for pharmaceutical R&D

# Perspective of lower income countries (LICs)

- Pharmaceutical prices of higher income countries are not affordable in LICs
- Local value judgement can facilitate (confidential) managed entry agreements
- There is even greater need to extend the evaluation framework in LICs
- Evidence generation is never designed according to the specific needs of LICs, this is also true for patient centric and societal criteria
- Deliberative processes may be manipulated both by political leaders and pharma companies
- Corruption in pharmaceutical pricing & reimbursement decisions can be prevented by
  - standard definition for each value criterion
  - explicit weights
  - explicit decision rule(s)

**Aggregate weights  
of additional  
value elements  
in lower income  
countries:  
personal opinion**

