

From innovation to patient care. The importance of cost-effectiveness analysis when making political decisions related to the innovation implementation

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Development of a new pharmaceutical is like building a new bridge
Average costs 12 billion SEK (2011)
or \$2.6 billion – 22 billion SEK (2015)

Costs 20 billion SEK (2000)



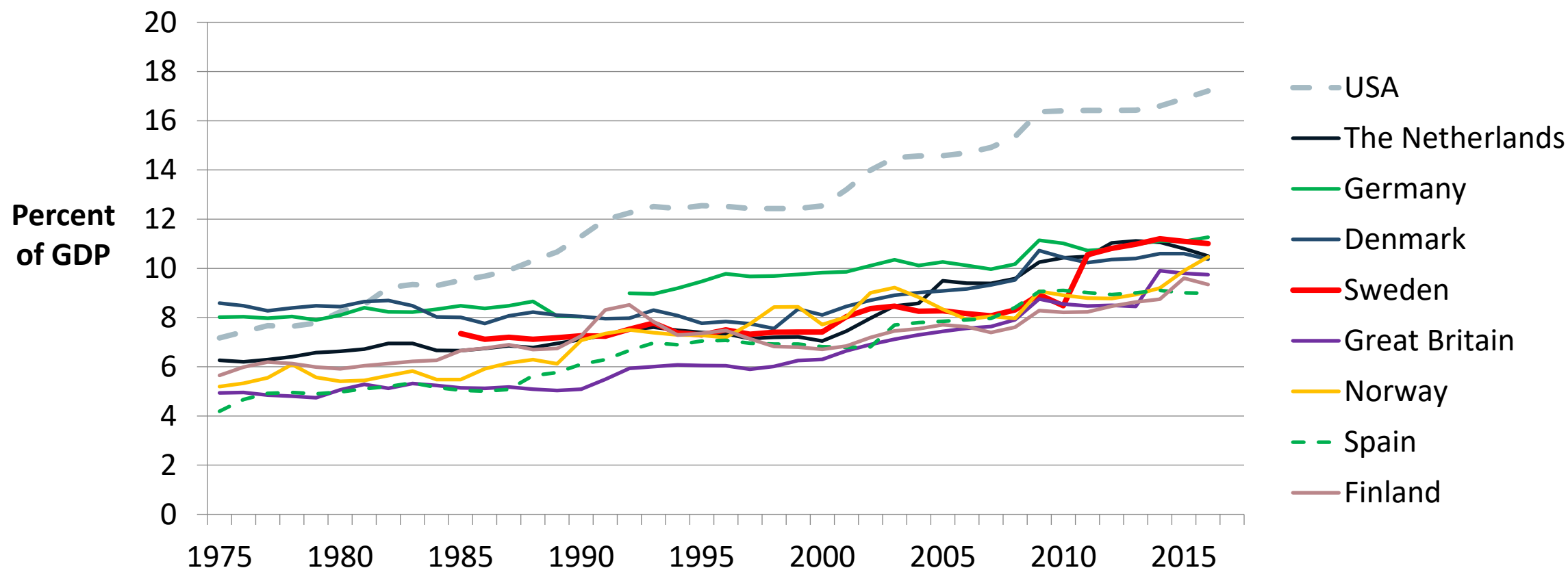
**Price to pass the bridge (treatment) pay for the bridge
(drug) but has nothing to do with the costs**

**Annual costs 300 million SEK
Annual revenue 1 000 million SEK**



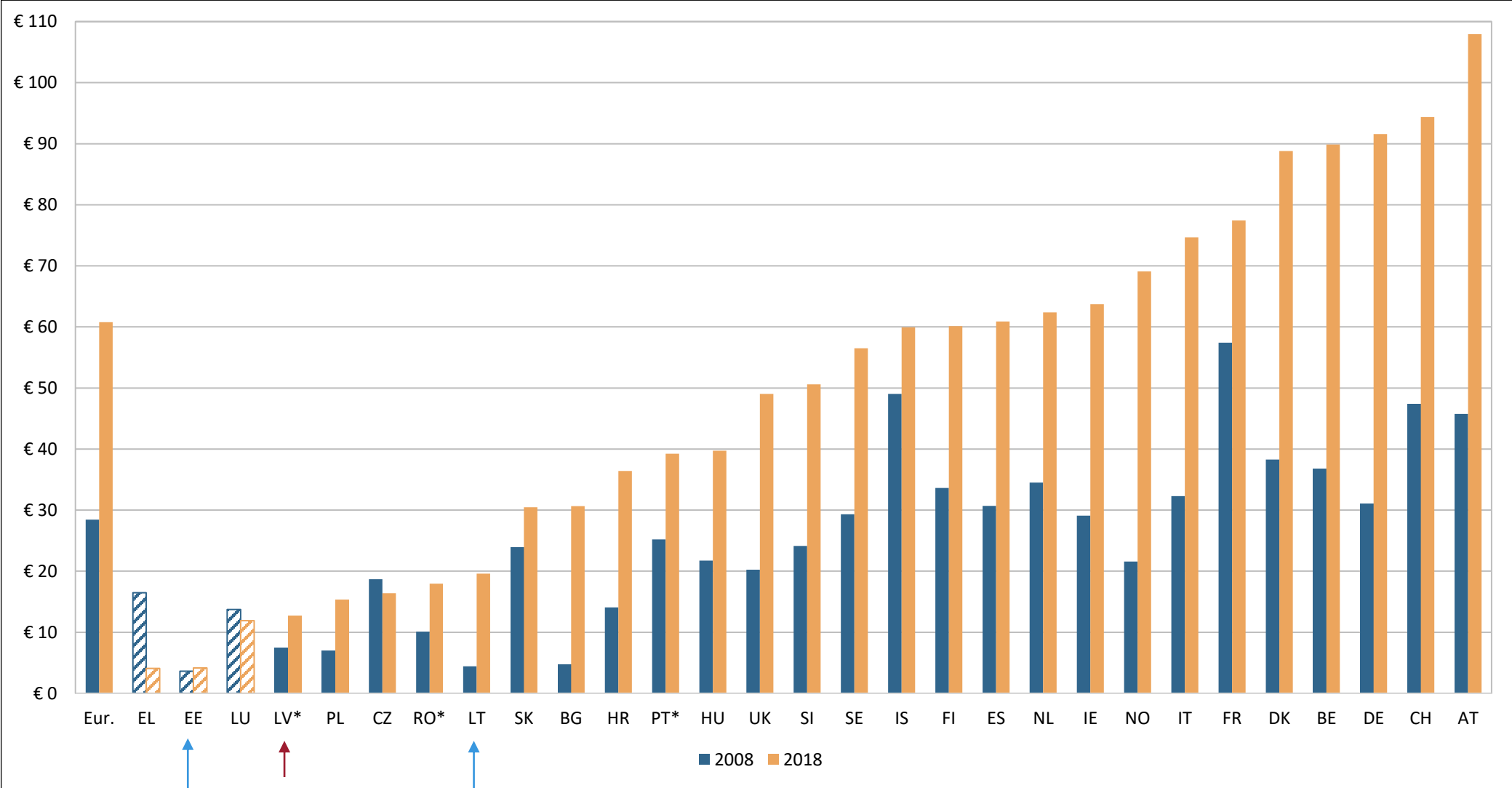
Health care expenditure 1975-2016

Percent of Gross Domestic Product, GDP



Source: OECD Health Statistics 2018 (www.oecd.org)

Cost of cancer medicines per capita (in 2018 price levels and exchange rates), 2008 & 2018



Source: Hofmarcher, T., Brådvik, G., Svedman, C., Lindgren, P., Jönsson, B., Wilking, N. (2019) Comparator Report on Cancer in Europe 2019 – Disease Burden, Costs and Access to Medicines. IHE Report 2019: Number. IHE: Lund, Sweden. (Forthcoming)



Health economics

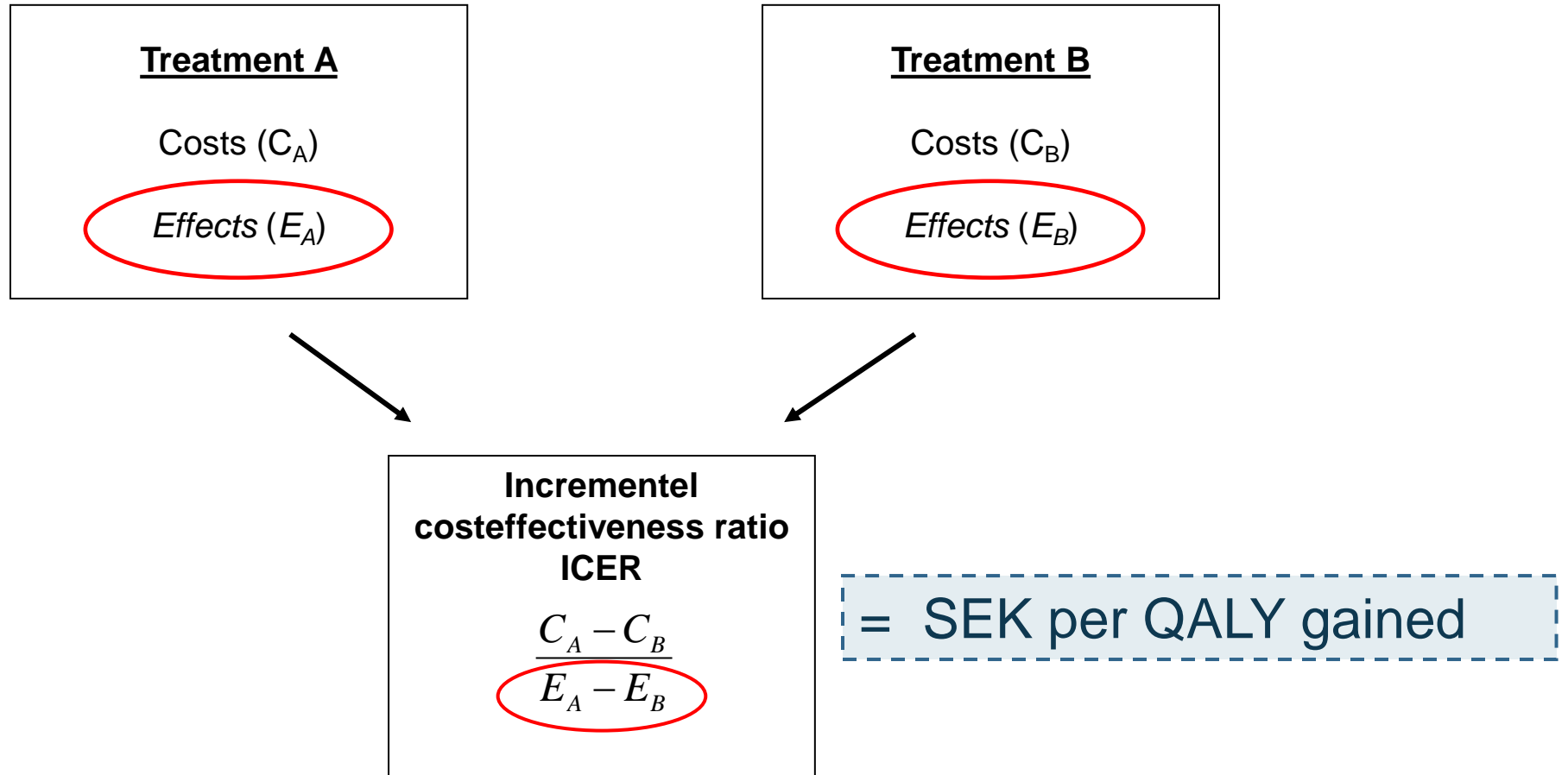
Economic theory applied in health care

For example, Health Economics may inform decision-making in health care:

- Provide information on resource usage using market-prices (SEK, £, \$, €)
- Provide information on utilities related to gain in life extension and quality of life, using hypothetical markets and study individuals behaviour to estimate Quality Adjusted Life Years (QALY)



Comparing treatments – Costs and health consequences



Consequences in a social economic perspective

Other pharmaceuticals

Outpatient care

Inpatient care

Social services (home care, rehabilitation)

Value of lost production

Life expectancy

Quality of life

Relationship between costs and Quality Adjusted Life Years gained (QALYs)

Pricing

Value Based Pricing (VBP) or Cost-Plus Pricing?

Cost-plus pricing:

1. Lead to wrong incentives, in that the higher the R&D costs, the higher the price
2. Investment costs for medicines that eventually do not make it to the final stage must be covered. A perverse situation if a company with many of such failures could justify a higher price
3. Cost plus pricing does not sufficiently encourage true innovation or benefit to patients, it does not reward value.

Health Technology Assessment (HTA) organizations in Europe

- National Institute for Health and Clinical Excellence (NICE) – England, Wales
- Federal Joint Committee (G-BA)– Germany
- The Haute Autorité de santé (HAS) - or French National Authority for Health – France
- Scottish Medicines Consortium (SMC) – Scotland

Dental and Pharmaceutical Benefits Agency (TLV) in Sweden

Decide reimbursement and establish price for drugs

- within the national benefit scheme for prescription drugs

Based on application from manufacturer or own initiative

Decisions based on 3 principles:

- Equal value of all human beings
- Need & solidarity
- Cost-effectiveness

Product-oriented



Reimbursement decisions on Pharmaceuticals from TLV/LFN 2002 – 2014

Year	Reimbursement without limitation	Reimbursement with limitation	Reimbursement denied	Total number of decisions
2002 Oct-	-	-	-	-
2003	16 (55 %)	4 (14 %)	9 (31 %)	29
2004	56 (89 %)	2 (3 %)	5 (8 %)	63
2005	51 (88 %)	3 (5 %)	4 (7 %)	58
2006	54 (73 %)	7 (9 %)	13 (18 %)	74
2007	41 (62 %)	11 (17 %)	14 (21 %)	66
2008	31 (30 %)	37 (36 %)	36 (35 %)	104
2009	21 (36 %)	22 (37 %)	16 (27 %)	59
2010	29 (41 %)	23 (33 %)	18 (26 %)	70
2011	62 (70 %)	20 (22 %)	7 (8 %)	89
2012	43 (63 %)	16 (24 %)	9 (13 %)	68
2013	40 (69 %)	12 (21 %)	6 (10 %)	58
2014	43 (51%)	33 (39%)	9 (10%)	85
Summary	467 (59 %)	190 (23 %)	146 (18 %)	823

Challenges

1. “Cost effective but not affordable”
2. Gen- and cell- therapies & individualized treatment (short treatment – long benefit – how can we reduce uncertainty?)
3. Value of drug treatment vary by patient - but only one single drug price. New pharmaceuticals are increasingly being developed for use across multiple indications.
4. Vaccines (silo perspective -important consequences beyond health care)
5. Antibiotics (we will not use them, but we will support innovations)

1. Cost-effective but not affordable

Affordability – “the new frontier” (Towse and Mauskopf 2018)

High upfront cost for uncertain lifelong benefits

Current budget system structured for chronic treatment.

Example hemophilia:

- Present value of lifetime treatment ~ \$8 million
- Price for a curative gene therapy ~ \$4 million
- Cost saving (or Cost effective) – but not affordable

Underinvestment compared to social optimum

2. Gene- and cell therapies – price per treatment

Kymriah	CAR T, acute lymphatic leukemic (ALL)	475,000 US dollar
Yescarta	Beta-cell lymphoma	373,000 US dollar
Luxturna	Inherited retinal dystrophy	425,000 US dollar per eye
Zolgensma	Spinal muscular atrophy (SMA)	2,1 million US dollar
Alofisel	Complications of Crohn's disease	67,000 US dollar
(Zynteglo	Transfusion dependent β -thalassemia	1,8 million US dollar)
Valrox*	Haemophilia, gene therapy	1,5 -3,0 million US dollar

*Valrox=valoctocogene roxaparvovec, under development

Performance-Based Risk-Sharing Agreements (PBRSA) in the US

Advantages:

1. Reducing risk to payer of making suboptimal purchase
2. Providing earlier access to medication for patients
3. Generating evidence on what works in real world

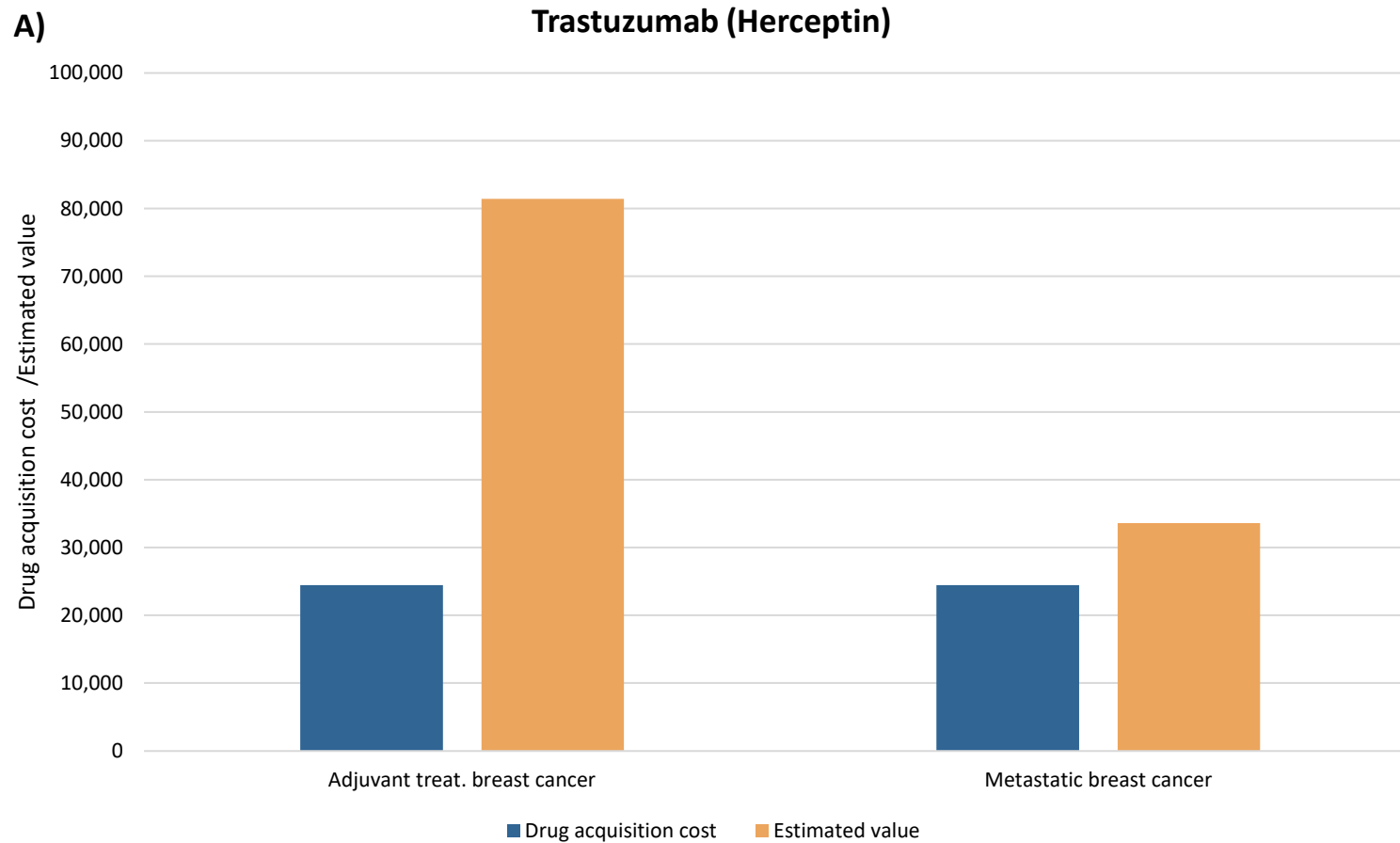
Barriers:

1. Additional efforts
2. Inadequate data structure
3. Significant costs of adjudication
4. Challenges in measuring relevant outcomes

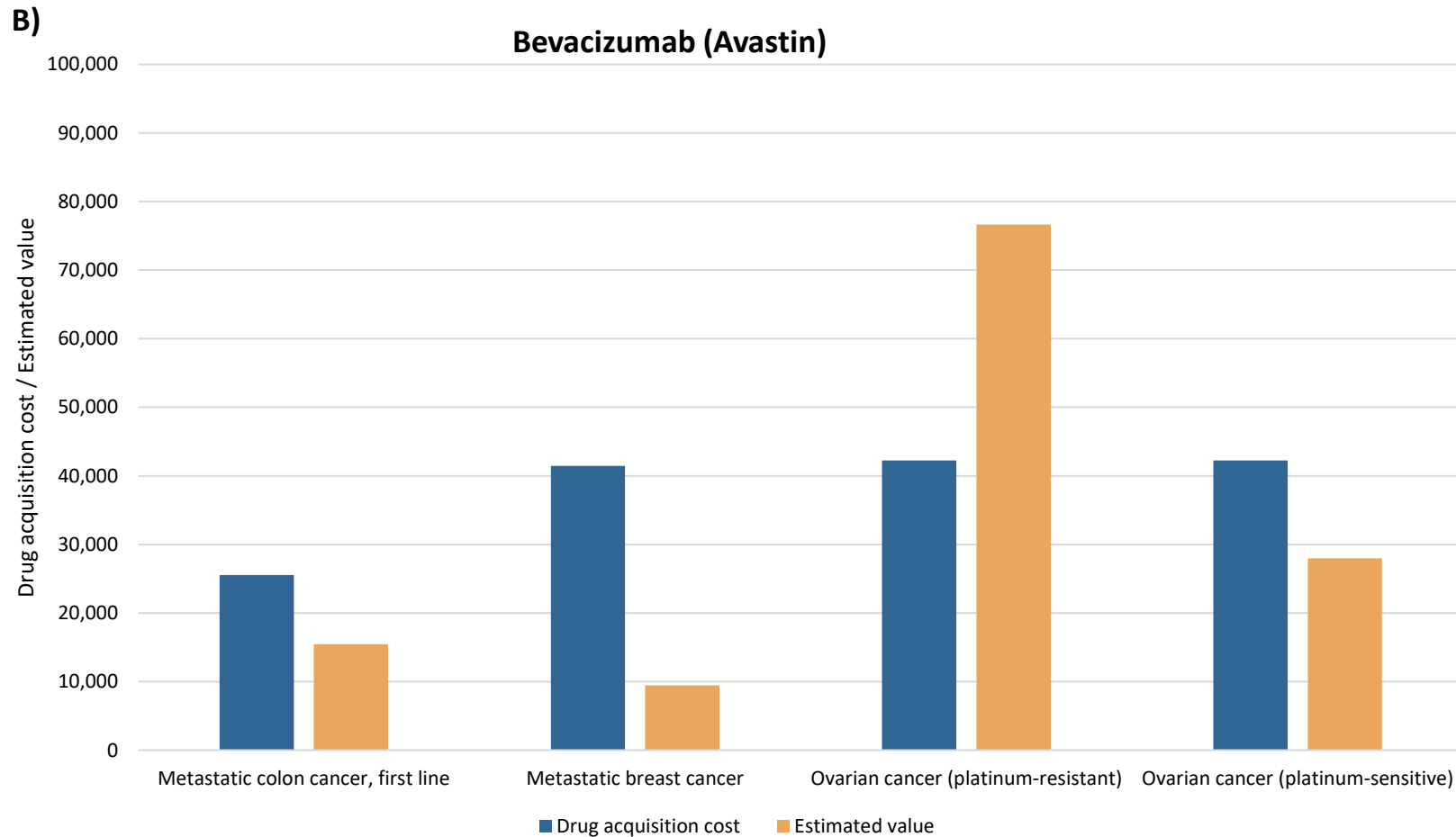
Source: Garrison et al, Am J Manag Care 2015.



3. Value of drug treatment varies by indication

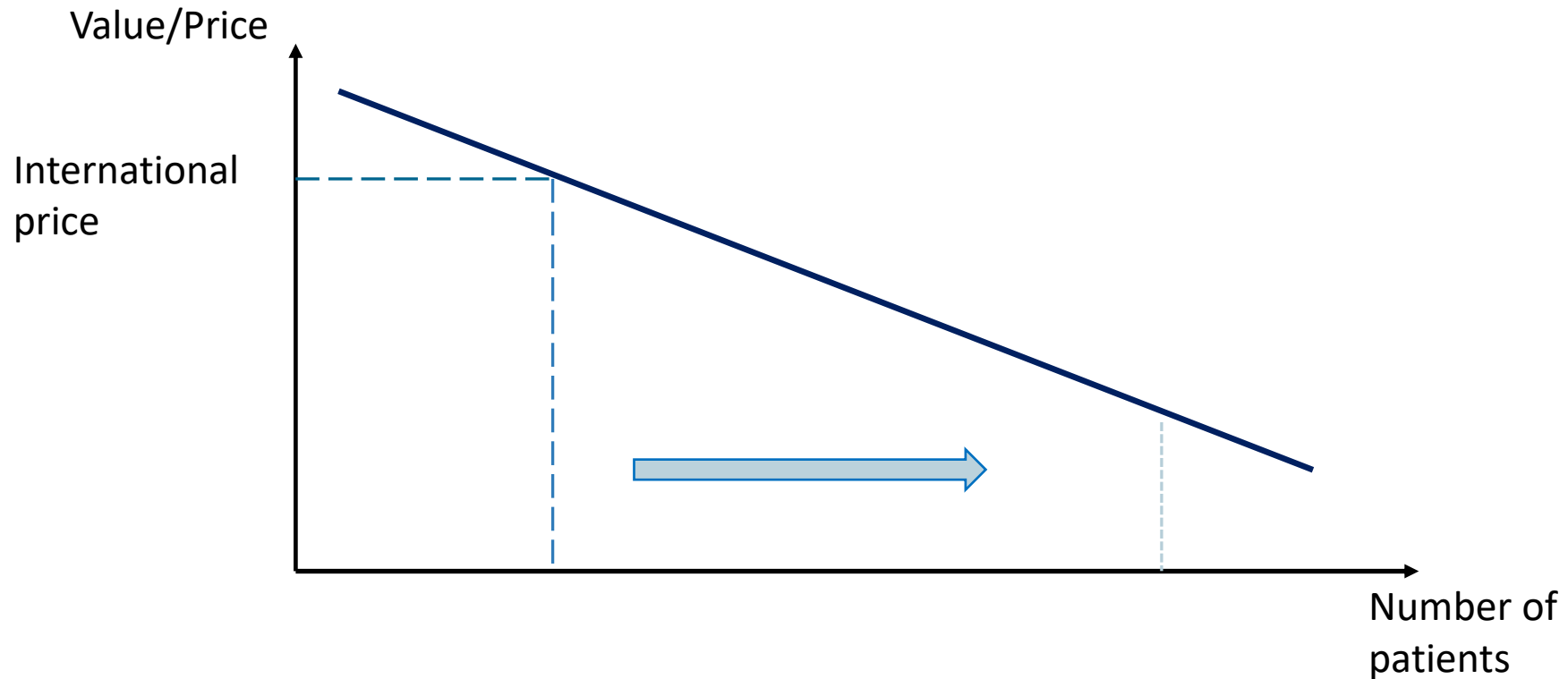


Value varies by indication, product-pricing (price per mg)



Why is it time for a new payment model for pharmaceuticals?

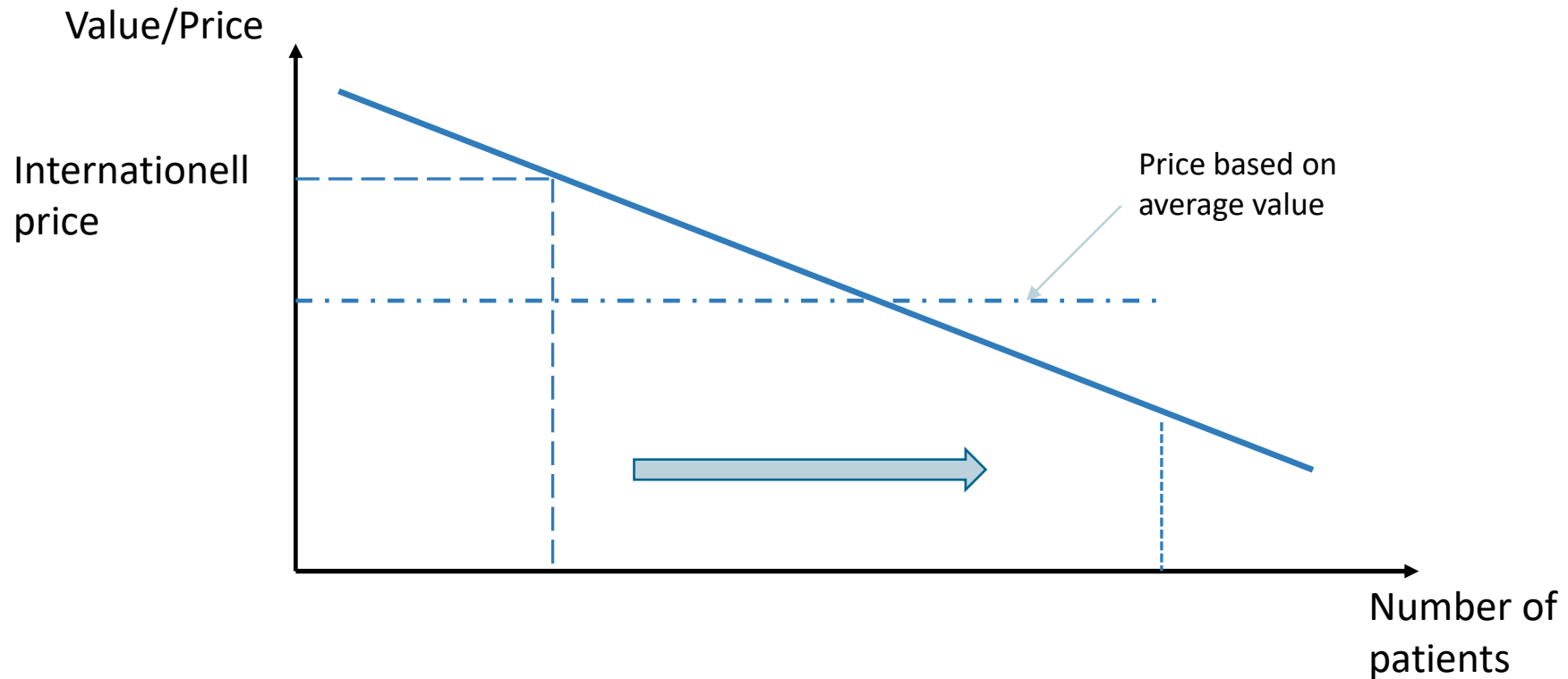
Value varies by indication. With a high international price reimbursement may only be granted for a small subpopulation even if the drug is approved for a larger patient population. The value of the drug will not be delivered if the drug is not used.



Is the current payment model optimal or should we develop new payment models?

Value varies by indication. Can price be based on average value?

Data on prescriptions per indication is required.



4. Vaccines (silo perspective - consequences beyond health care)

Estimated value of vaccinating the Swedish population against COVID-19 assuming avoidance of 3 months with a new wave of the pandemic

Health economic consequence	QALY gain	Monetary gain*
Health gain vaccinated individual	24,000 QALYs	€1.2 billion
Macroeconomic impact		€7.5 billion
Quality of life in the general population	63,000-132,000 QALYs	€3.2-6.6 billion
TOTAL		€11.9-15.3 billion

Source: Persson U, Olofsson S, Althin R, Palmborg A, Dorange AC. Acceptance and Application of a Broad Population Health Perspective when Evaluating Vaccine, Vaccine 2022.

6. Antibiotics (we will not use them, but we will support innovations)

Value of new medicines

Traditional value drivers

Cost savings in the health care for the treated

Health gain (QALY) for the treated individual

(Productivity gain for the treated)

Additional value drivers antibiotics

Transmission value (benefits of avoiding the spread of infection)

Enablement value (value associated with enabling other treatments or procedures, e.g. surgery and chemotherapy)

Insurance value (value of having a treatment available in case)

Diversity value (preserving the efficacy of existing antibiotics)

Spectrum value (reduce the spread of antimicrobial resistance)

Novel action value (spillover effects from a new mechanism of action)

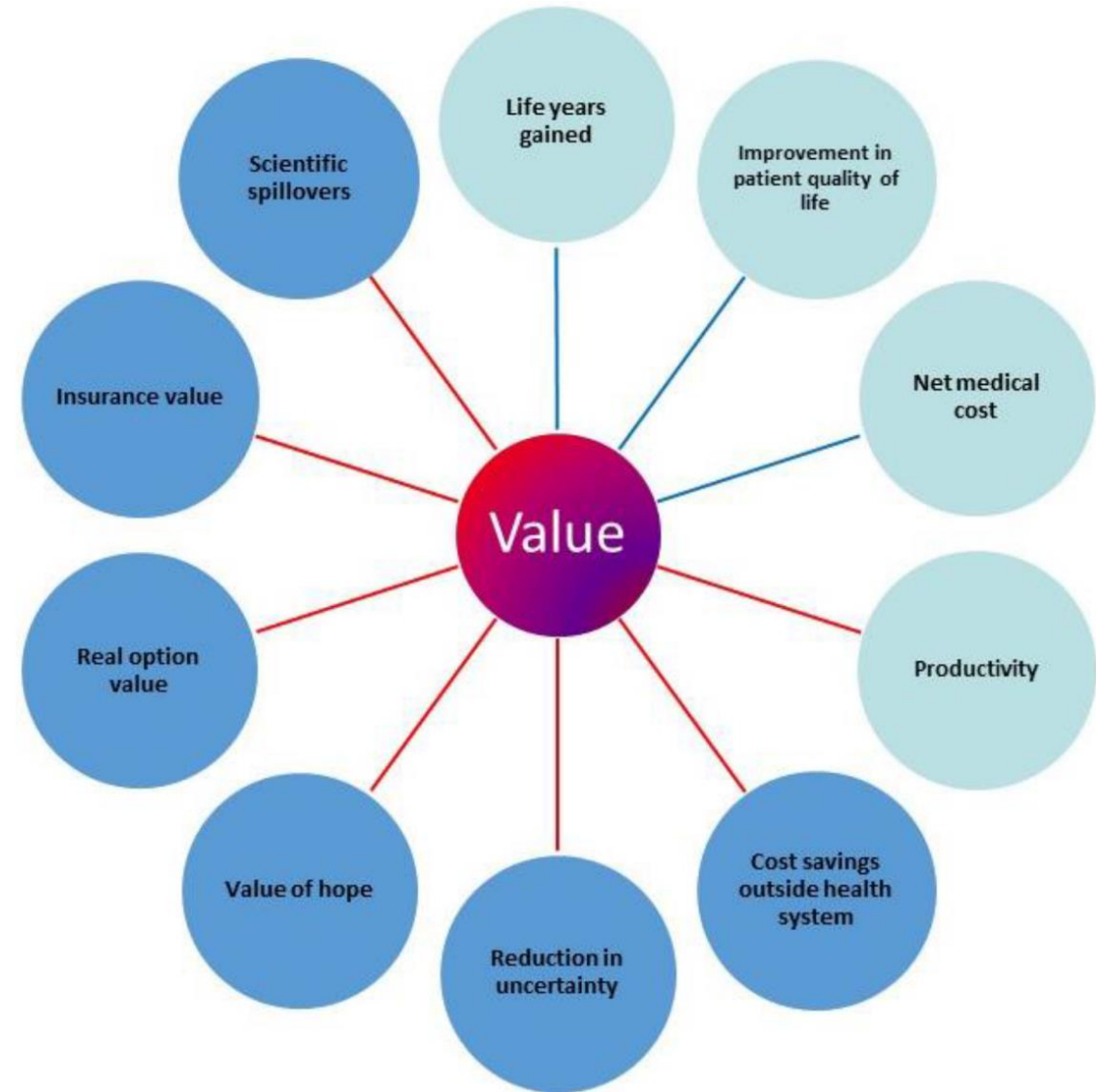
Ref: Karlsberg Schaffer et al. 2017 (OHE)

Proposed elements of Value

Source:

Garrison, L.P.; Towse, A. Value-Based Pricing and Reimbursement in Personalised Healthcare: Introduction to the Basic Health Economics. *J. Pers. Med.* **2017**, *7*, 10.

<http://www.mdpi.com/2075-4426/7/3/10>



“ISPOR Value Flower” (Lakdawalla et al. 2018)

Elements of Value

Challenge: Map each element into an underlying economic framework for value assessment.

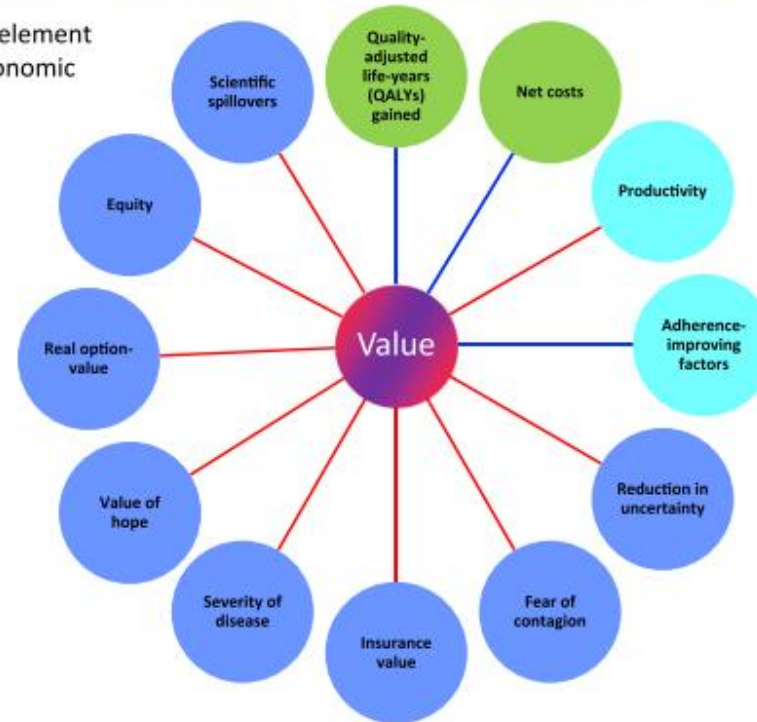


Fig. 1 – Elements of value. Note. Green circles: core elements of value; light blue circles: common but inconsistently used elements of value; dark blue circles: potential novel elements of value; blue line: value element included in traditional payer or health plan perspective; and red line: value element also included in societal perspective.

Challenges & the way forward

1. Affordability problem
 2. Short treatment- long benefit
 3. Multiple indications
 4. Vaccines
 5. Antibiotics
1. Broad societal perspective, flexible budgets
 2. Manage enter agreements & pay for performance
 3. Indication based pricing
 4. Broad societal perspective
 5. Consider new additional value drivers

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