How do we measure VALUE? Different models and challenges in pricing

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What is a fair price for a medicine?

- “A fair price is one that is **affordable** for health systems and patients and that at the same time provides sufficient **market incentive** for industry to invest in innovation and the production of medicines.”

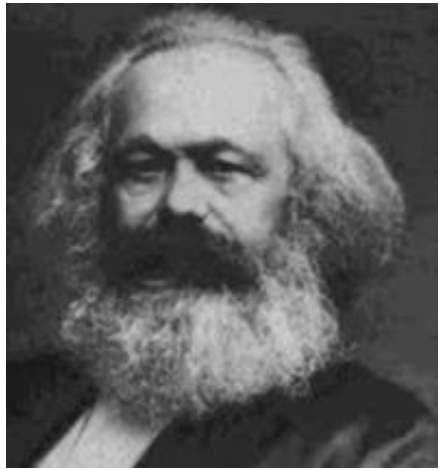
- NB – pharma **DO** need rewarding for providing innovation

World Health Organization

Different ways to look at pricing

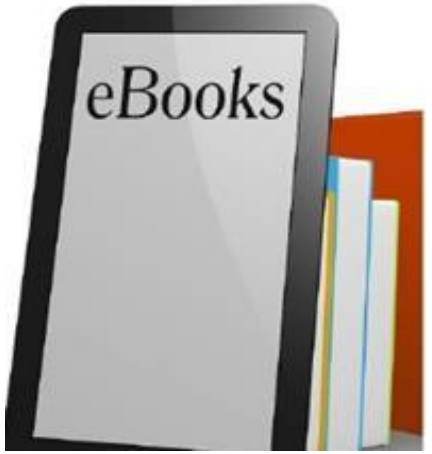
“Four Reasons Drugs Are Expensive, Of Which Two Are False”, Jack Scannell*

Inputs (costs)



Value

Power



Prize / Incentive

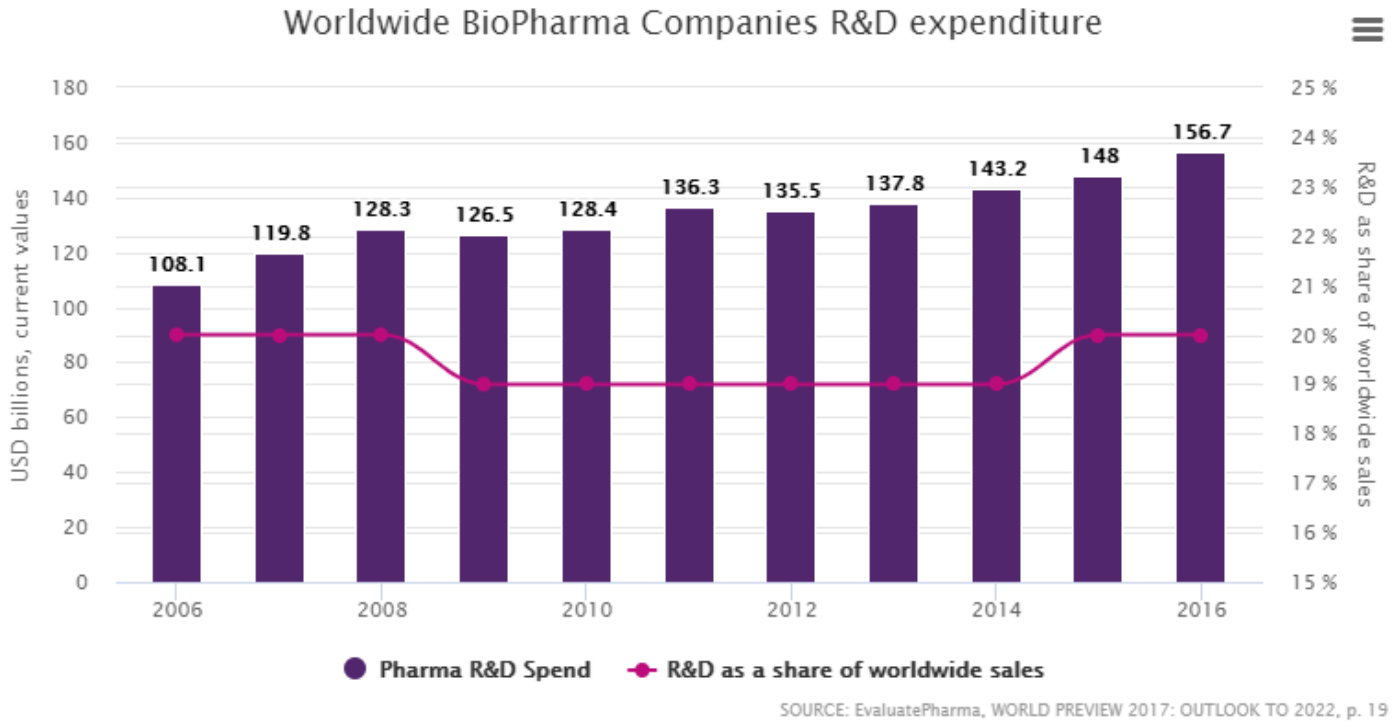
*Based on Article in Forbes, Oct 13, 2015: https://www.forbes.com/sites/matthewherper/2015/10/13/four-_reasons-drugs-are-expensive-of-which-two-are-_false/#384a3ef44c3b

Cost-based pricing: How much do pharma spend on R&D?

Inputs (Costs)

- *Pharma view: false to determine the price of a medicine, but appropriate to explain overall company profitability.*
- *Efficiency: too much health spend is off-target*

- ZPW View:
  - Costs are not an appropriate way to determine the price of a medicine, but are relevant across the industry
  - Because cost-based pricing rewards innovation but also 'me-too' drugs
  - It is fair to charge a return for investment in R&D
  - Too much pharma spend is inefficient and 'off-target'

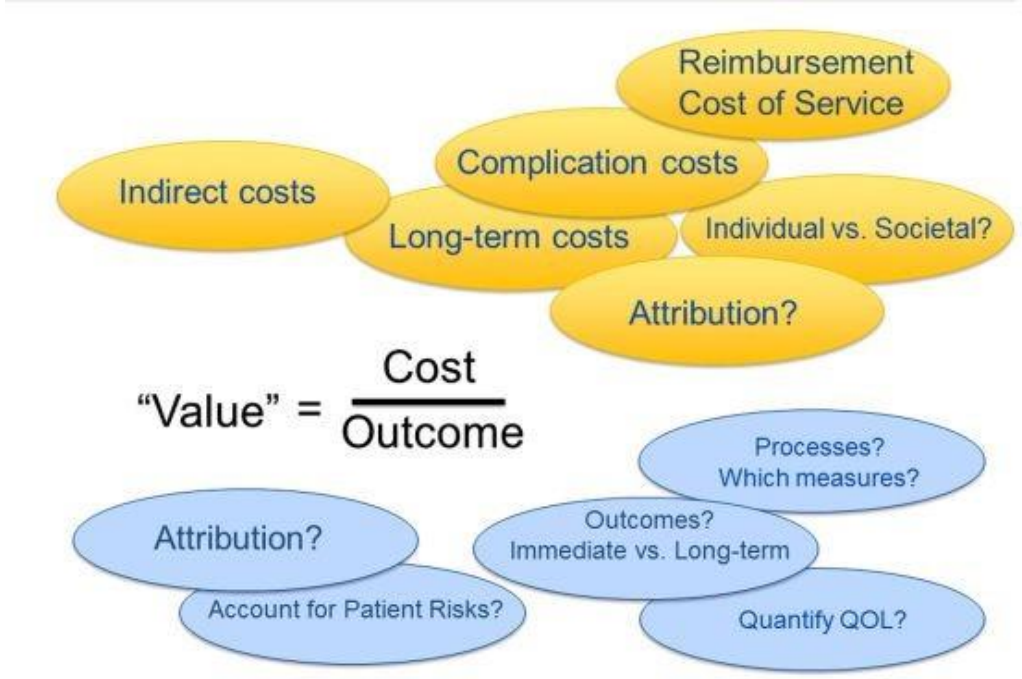


SOURCE: EvaluatePharma, WORLD PREVIEW 2017: OUTLOOK TO 2022, p. 19

Value-Based Pricing?

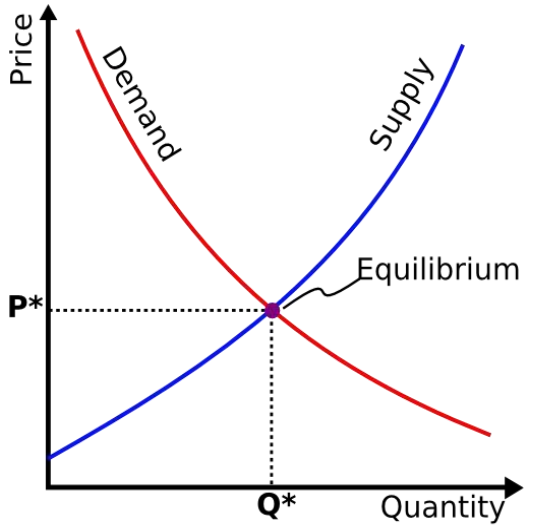
- "Price = amount of perceived value the consumer gets"
- Scannel argues value-based pricing evolved as a way of charging customers more (e.g. luxury goods)
- Pharma view: "Allows for objective justification of a price – despite evidentiary uncertainty."
- Outcome?
  - What outcomes matter? Who determines value? Patient? Public? Payer?
  - Heath-related? Wider societal benefits?
  - Certainty of data?

$$\textbf{Value} = \frac{\textbf{Cost}}{\textbf{Outcome}}$$



Power Pricing – Is there competition?

- JS describes this as the “exercise of intellectual property rights, to create scarcity and to find the maximum price that the market will bear”
- Example: Martin Shkreli who raised the price of HIV drug by a factor of 56 from $13.50 to $750 per pill
- Example: alemtuzumab from leukaemia to multiple sclerosis and “list price rise of over one thousand – yes, one thousand – percent” (Scannel, Forbes)
- Pharma view: “*Fierce competition amongst patented drugs drives pricing behavior too”*
- During the patent period many drugs operate a monopoly within an indication (at least for a time period). This ceases when the patent expires and generics enter the market



Incentive Pricing – Reward for Gamble - Rare Conditions

- *Pharma view: appropriate, particularly in case of market failure (e.g. orphans, neglected diseases)*
- Should we assign additional value to medicines for rare conditions?
- EMA Orphan Status:
  - disease that is life-threatening or chronically debilitating
  - the prevalence of the condition in the EU must not be more than **5 in 10,000** or it must be unlikely that marketing of the medicine would generate sufficient returns to justify the investment needed for its development
  - medicine must be of significant benefit to those affected by the condition.
- Orphan = 10 years of market exclusivity from similar medicines with similar indications
- HTA: different willingness to pay thresholds? E.G. HST

NICE QALY Thresholds	
NICE Process	**QALY Threshold**
Standard	20,000 – 30,000
End of Life	50,000
HST	100,000 – 300,000

NICE Highly Specialised Technologies	
Incremental QALYs gained (per patient, using lifetime horizon)	**Weight vs 100k/QALY**
≤ 10	1
11 - 29	1 - 3
≥ 30	3

Pharmaceuticals is one of the most profitable sectors

Table 4.3. Ranking of top 15 industrial sectors by overall one-year sales growth and related data for the EU, US and Japanese companies in the 2014 *Scoreboard*.

Rank	Sector	World-wide Sales growth 1y (%)	EU-633		US-804		Japan-387	
			Sales growth 1y (%)	Profit.*	Sales growth 1y (%)	Profit.*	Sales growth 1y (%)	Profit.*
1	Construction & Materials	7.9	-0.1	6.6	7.2	10.4	9.3	4.1
2	Automobiles & Parts	7.8	1.8	6.2	4.9	4.8	16.1	7.3
3	Software & Computer Services	7.2	4.3	17.3	6.7	22.0	5.0	3.8
4	Health Care Equipment & Services	7.0	1.3	14.6	8.7	9.4	9.7	9.8
5	Electronic & Electrical Equipment	6.5	1.0	8.5	2.2	11.9	12.1	5.9
6	General Industrials	4.9	1.7	7.1	2.6	12.7	10.5	2.3
7	Aerospace & Defence	4.3	4.3	7.1	3.4	11.0	15.3	6.2
8	Leisure Goods	4.5	6.2	21.5	3.2	12.9	4.5	4.0
9	Chemicals	3.9	-0.7	9.9	3.9	12.8	12.0	5.4
10	Pharmaceuticals & Biotechnology	2.8	0.3	16.7	1.6	21.9	8.4	9.9
11	Technology Hardware & Equipment	2.2	-6.2	4.9	1.5	16.3	13.7	8.9
12	Industrial Engineering	1.4	-0.5	7.2	-2.3	11.5	12.6	5.8
13	Oil & Gas Producers	0.0	-3.4	7.8	-6.1	11.6	11.9	1.4
14	Fixed Line Telecommunications	-2.6	-6.6	13.7	1.1	23.5	2.1	11.1
15	Banks	-3.2	-5.7	3.2				
	Total 40 industries	**2.8**	**-2.0**	**6.9**	**2.0**	**13.9**	**11.5**	**5.7**

** Profitability: operating profits as percentage of net sales.*
Source: The 2014 EU Industrial R&D Investment Scoreboard. European Commission, JRC/DG RTD

- Scannel: "The drug industry has higher profit margins and higher R&D intensity than any other industry."

Profits:

- EU: Software & Computer Services (17.3%) and Pharmaceuticals & Biotechnology (16.7%).
- US: Software & Computer Services (22.0%) and Pharmaceuticals & Biotechnology (21.9%).
- Japan: Pharmaceuticals & Biotechnology (9.9%), the highest profitability sector

R&D

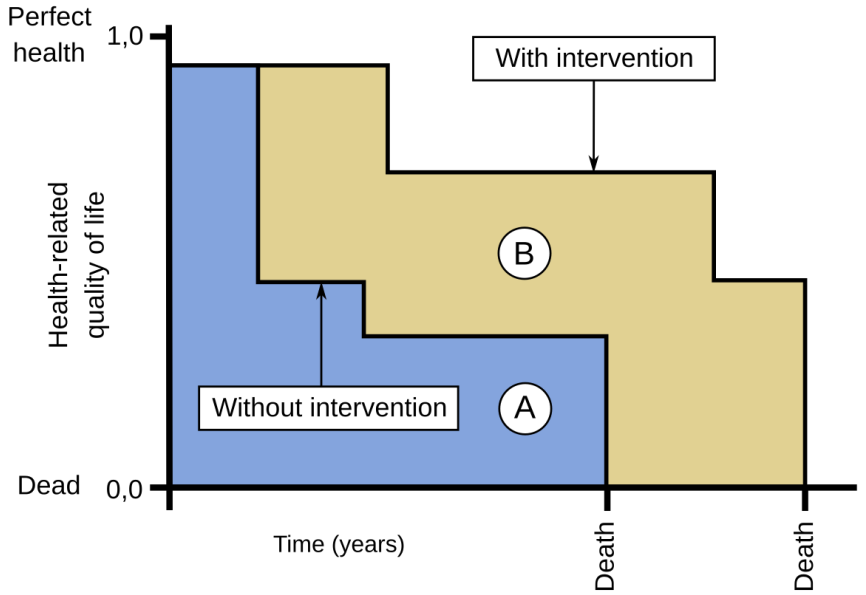
- Pharmaceutical sector ranks top in terms of R&D at nearly 100 billion euro

- **EU Industrial R&D Investment Scoreboard - 2014**

http://iri.jrc.ec.europa.eu/scoreboard14.html

Difference between affordability and cost-effectiveness

- Cost-effectiveness: value for money (cost v benefit)
  - QALY = Quality-adjusted life year
- Affordability: budget impact (total cost)
  - Voluntary Scheme for Branded Medicines Pricing and Access (VPAS)
  - ‘Budget Impact’ Test - £20 million threshold



Example: Zolgensma (spinal muscular atrophy)

- Potentially curative treatment, offers significant QALY gains (many years of potential benefit)
- “world’s most expensive therapy” - $2.1 million
- It may be cost-effective, but is it affordable?
- How does the system afford to pay for 275+ cell and gene therapies in development?

5,300 views | Jun 5, 2019, 10:32am

At Over $2 Million Zolgensma Is The World's Most Expensive Therapy, Yet Relatively Cost-Effective

Continuous Therapies v Fixed Treatment Duration

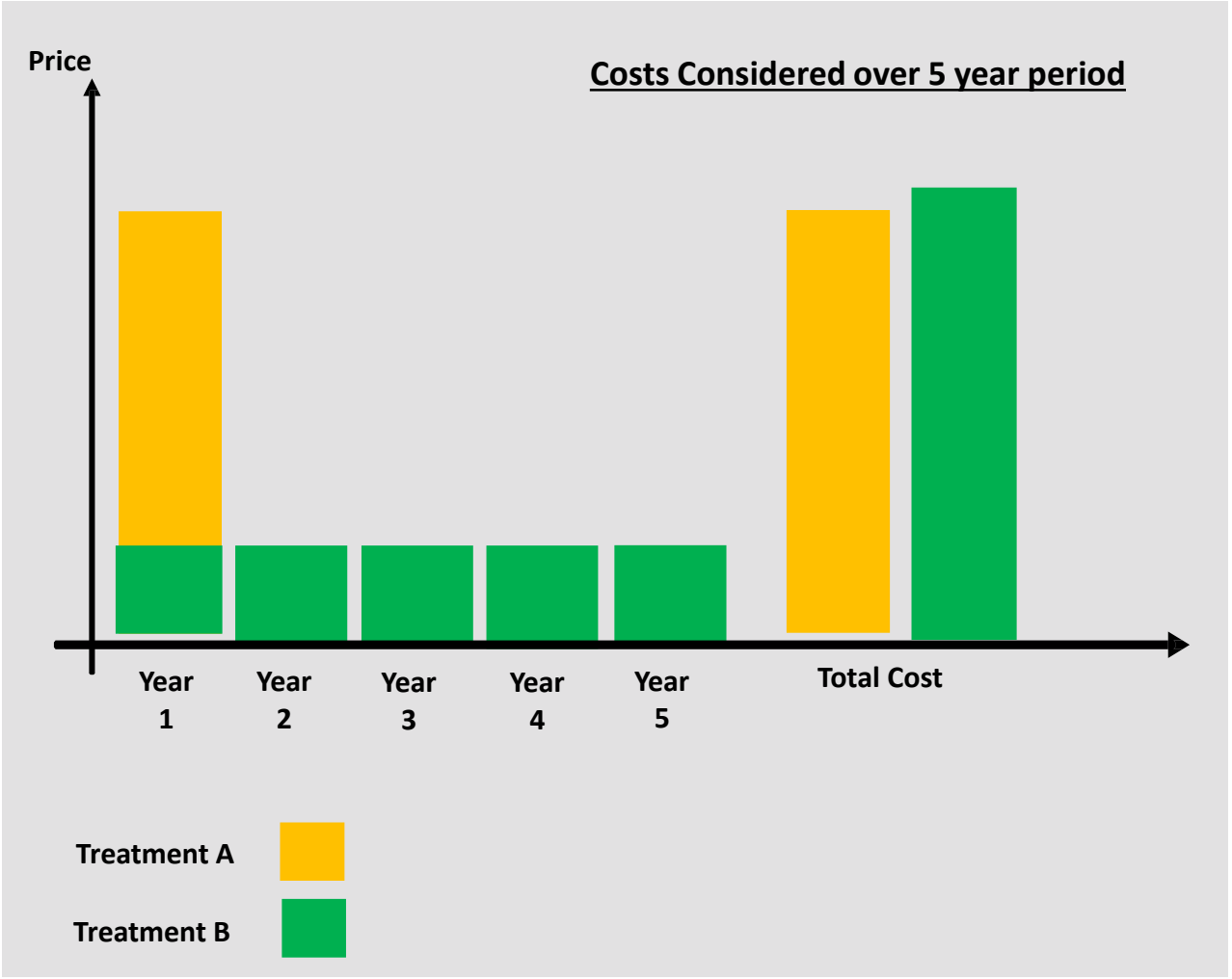
- Cost over what time period? How long does the data suggest the fixed-treatment duration is effective?

Example: Zolgensma (costs over 5 years)

- Zolgensma - Single infusion - $2.15 million
- Spinraza - Continuous treatment - $2.25 million over 5 years

- Turning cancer into a chronic condition (e.g. chronic myeloid leukaemia) – is this an affordability issue?

- Treatments that are delivered with a fixed treatment duration are more likely to be affordable long-term



Uncertainty

- Uncertainty in the clinical data (e.g. the benefit of a new treatment or the comparator treatment) makes it hard to determine the value
- Rapid access (for patients) v Long-term data certainty
- Particular problem where the drug is intended for a small patient population (e.g. rare disease or specific mutation)

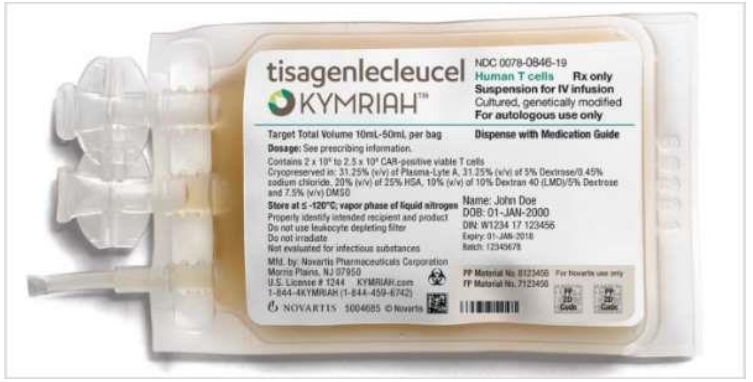
- Is there a need for new systems to enable 'conditional' or temporary access whilst further evidence is gathered to assess value?

THE CANCER DRUGS FUND

NICE highlighted "a lack of evidence" on the effect of the drug on overall survival

There was a great deal of uncertainty in the available evidence of clinical effectiveness compared to existing treatments

Potentially Curative Treatments?



- Potential for long-term benefit, but short-term data
- Huge amounts of uncertainty. How to share the risk?

1. Pay for Performance
   - ❑ Outcomes based payments linked to results in clinical practice
   - ❑ (e.g. using the Systemic Anti-Cancer Therapy database in NHS England)
2. Coverage with evidence generation
   - ❑ Temporary access granted to enable evidence generation, before a full-assessment takes place at a later date
   - ❑ (e.g. Cancer Drugs Fund)
3. Annuity or amortisation
   - ❑ Spread payment over time – like a mortgage - reduce upfront cost
   - ❑ e.g. Zolgensma
4. Lump-sum remuneration
   - ❑ Provide treatment to the whole patient population for a fixed cost
   - ❑ e.g. VPAS (whole drugs budget) or Orkambi (Vertex, Cystic Fibrosis)

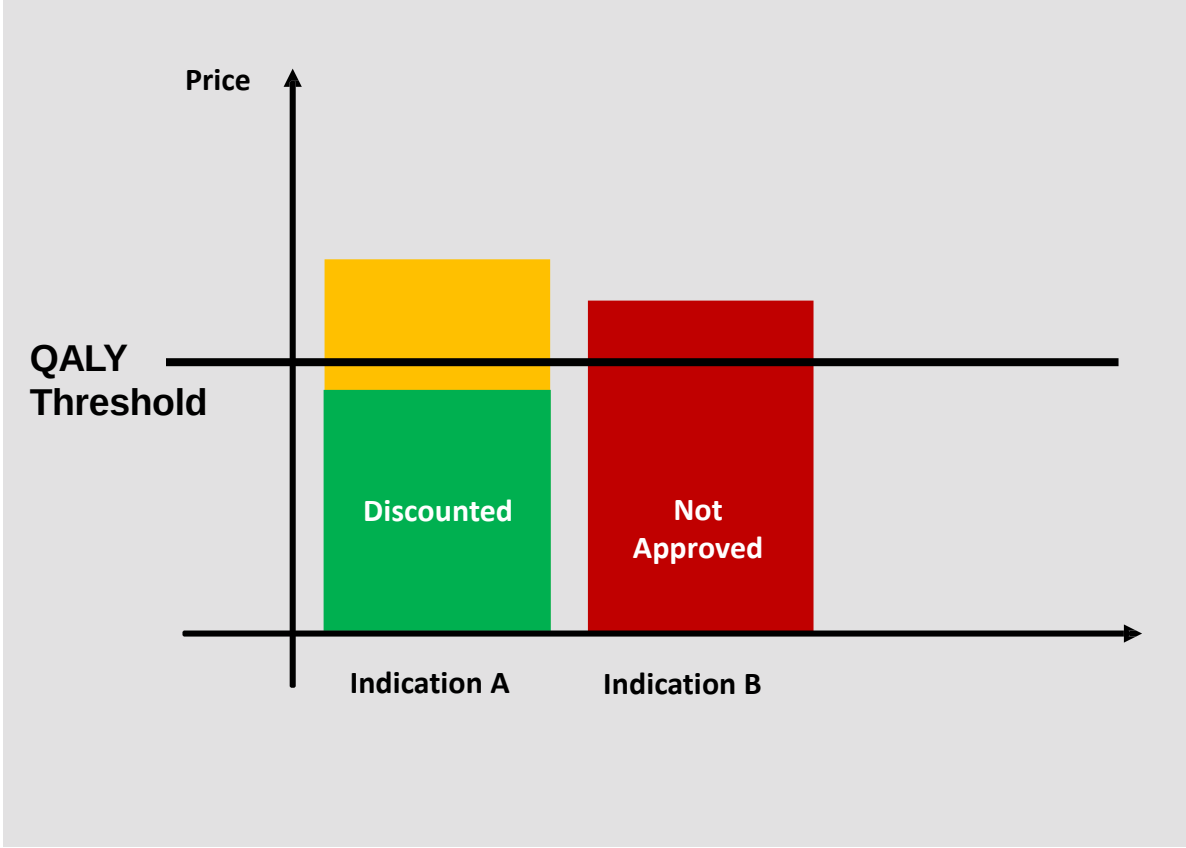
- In reality, whilst most of these address uncertainty in cost-effectiveness, they do little for affordability (unless the price is reduced as a result)

Multi-Indication Pricing

- Medicines may be effective in multiple indications, in each indication they may offer a different value
- If prices are intended to reflect the value a medicine brings, prices should be different across different indications to reflect their different values
- Many healthcare systems do not allow MIP
- Current systems are not equipped to handle this – e.g. pharmacy prescriptions?
- May be unfair for rarer conditions

Example:

- Indication A: Larger population of patients
- Indication B: Smaller population, assessed subsequently, no opportunity to adjust price



Combination Pricing – “not cost effective at zero price”

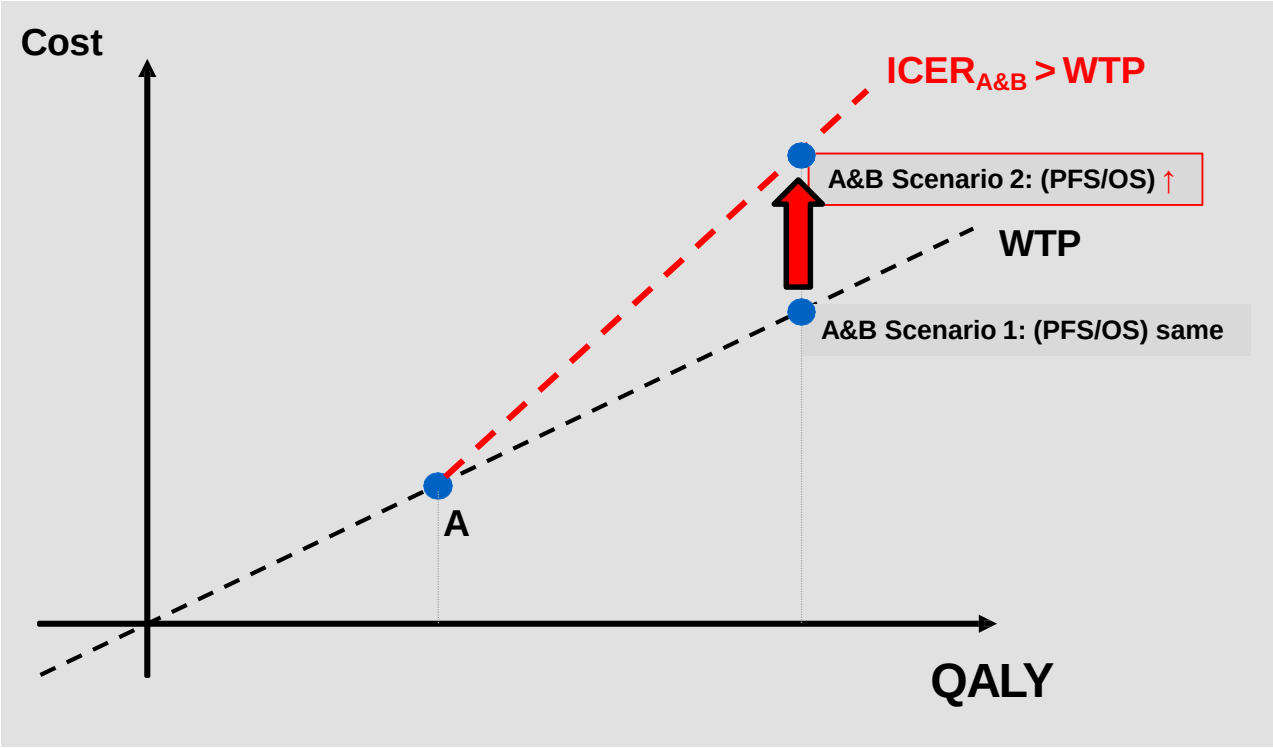


Image from Stefan Weber presentation at WECAN 2019

(EXAMPLE 2: THE COMBINATION EXTENDS TIME TO PROGRESSION, BUT WE ASSUME SURVIVAL DATA IS NOT (YET) MATURE

- This is a serious issue
- Shows the need for Multi-Indication Pricing

Devil’s Advocate:

- Does addition of combination therapy (concurrently) add any benefit to using treatments consecutively?

- Balance between rapid access and data certainty: OS data is immature
- Should we wait until OS data is mature?

End of Life?

- Should we assign additional value to End of Life treatments?
- In a system with finite health budgets, will patients with other health conditions suffer because we have paid more for end of life treatments?
- Difference between relative benefits and absolute benefits?
  - 3 month benefit / 6 month expected survival = 50%
  - 3 month benefit / 2 year expected survival = 12.5%

NICE QALY Thresholds	
NICE Process	**QALY Threshold**
Standard	20,000 – 30,000
End of Life	50,000

NICE has a higher threshold of up to £50,000 for End of Life treatments, with criteria:

Short life expectancy – normally less than 24 months

Extension to life – normally at least a further three months

Changing the mindset: ‘Simple COVID health economics’

Dr Amanda Adler, Chair of NICE technology appraisal committee B:

“Gel on the left ‘kills 99.99% of germs’, so is more effective than the right one which kills only 99.9%.

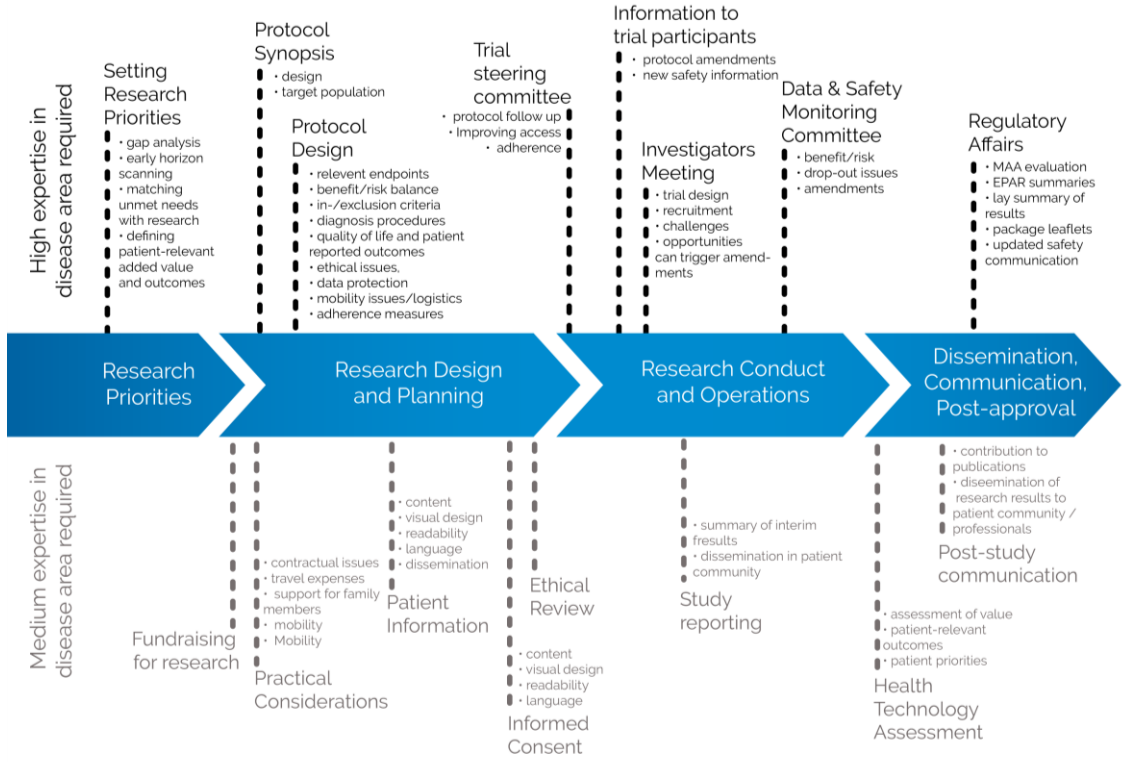
One might expect to pay more; but, the left gel is 79% the price of the other even correcting for volume.

Cheaper, more effective = dominant”

Image: https://twitter.com/DrAmandaAdler/status/1306293645297946625 (Last Accessed, 22/09/2020)

This goes beyond just HTA…

- Lots of opportunities for patient involvement in medicines R&D
- Lack of patient involvement in R&D influences HTA decisions
- We already have a roadmap…



Geissler et al (2017) - Improving Patient Involvement in Medicines Research and Development: A Practical Roadmap - https://journals.sagepub.com/eprint/6J5ErcVqeCi4BDdCE7FD/full

Summary:


Fair pricing requires a balance between affordability and rewarding innovation
Different ways to look at pricing, of which value-based pricing is probably the most appropriate
The pharmaceutical sector is highly profitable, but also invests highly in R&D
Current pricing and reimbursement systems facing serious issues