WECAN SmartStart in Cancer Patient Advocacy

WECAN MASTERCLASS in Cancer Patient Advocacy

# 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.”

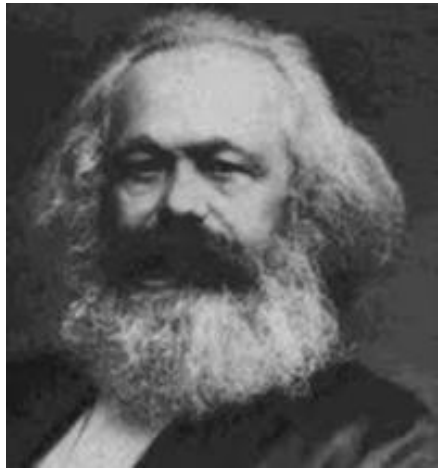
- NB – pharma **DO** need rewarding for providing innovation

World Health Organization

# Different ways to look at pricing

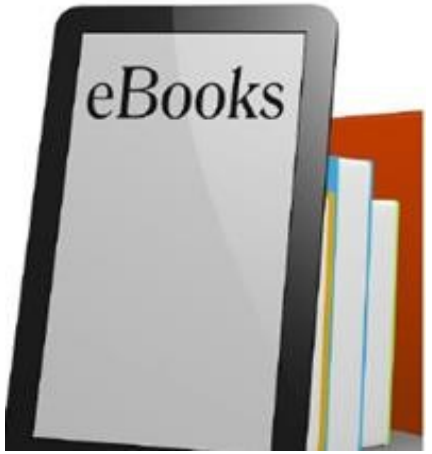
**“Four Reasons Drugs Are Expensive, Of Which Two Are False”, Jack Scannell***

**Inputs (costs)**



**Value**


**Power**




**Prize / Incentive**

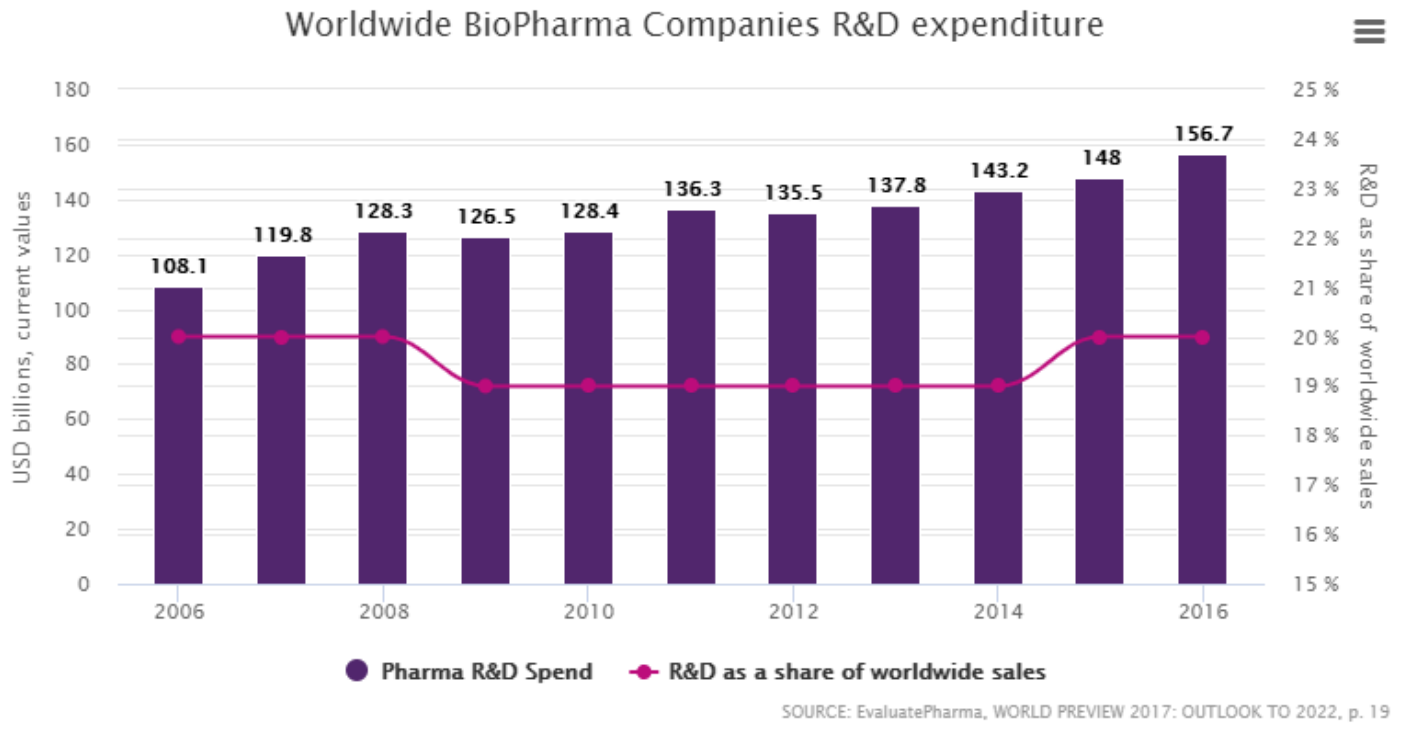

*Based on Article in Forbes, Oct 13, 2015:
https://www.forbes.com/sites/matthewherper/2015/10/13/four-reasons-drugs-are-expensive-of-which-two-are-_false/#384a3ef44c3b

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

**Inputs (Costs)**

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

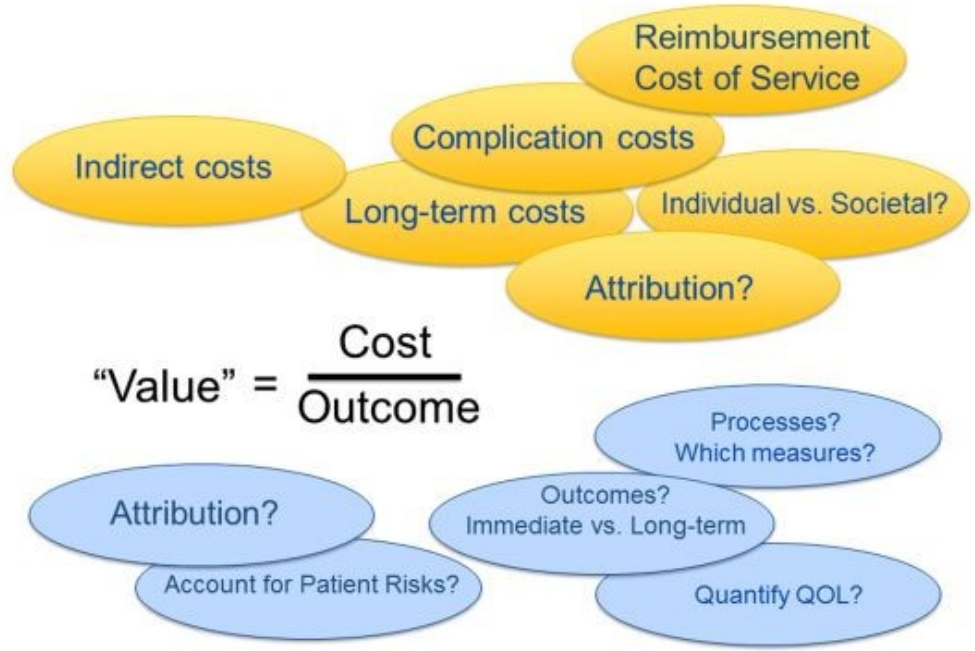
- ZPW View:
  - Costs are not an appropriate way to determine the price of a medicine, but are relevant across the industry
  - Because cost-based pricing rewards innovation but also ‘me-too’ drugs
  - It is fair to charge a return for investment in R&D
  - Too much pharma spend is inefficient and ‘off-target’



# 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)
- SW 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?

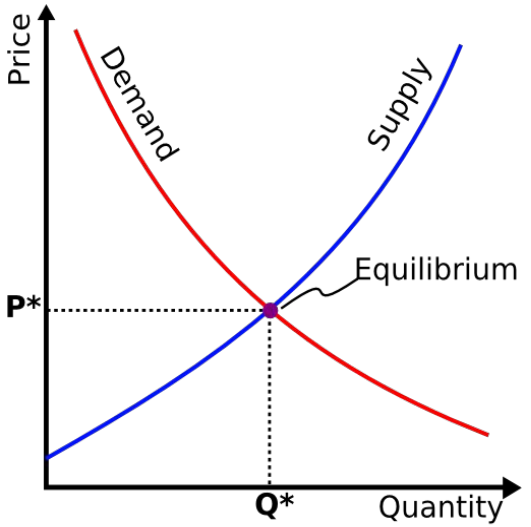
$$\text{Value} = \frac{\text{Cost}}{\text{Outcome}}$$




Ray et al (2016)

# 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)
- SW 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

- *SW 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**

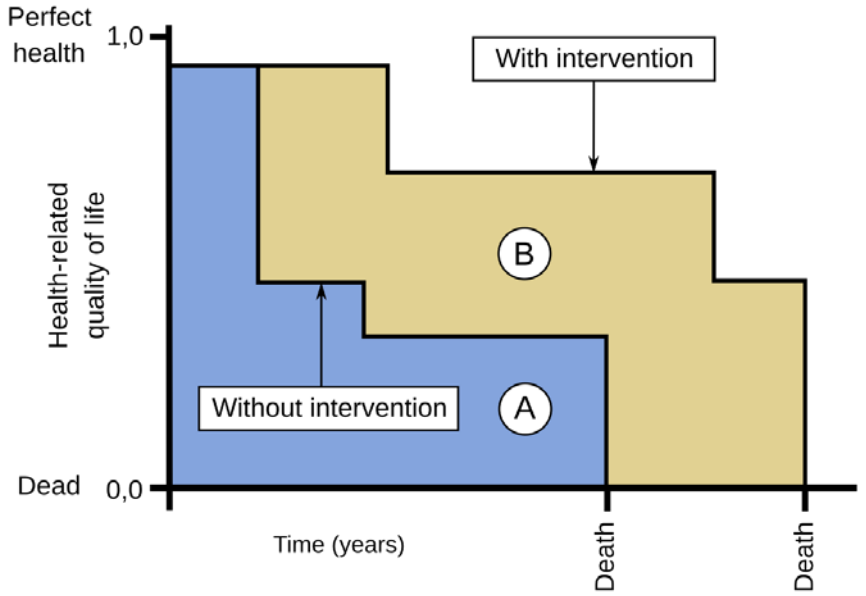
http://iri.jrc.ec.europa.eu/scoreboard14.html

# 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**

Forbes

# Continuous Therapies v Fixed Treatment Duration

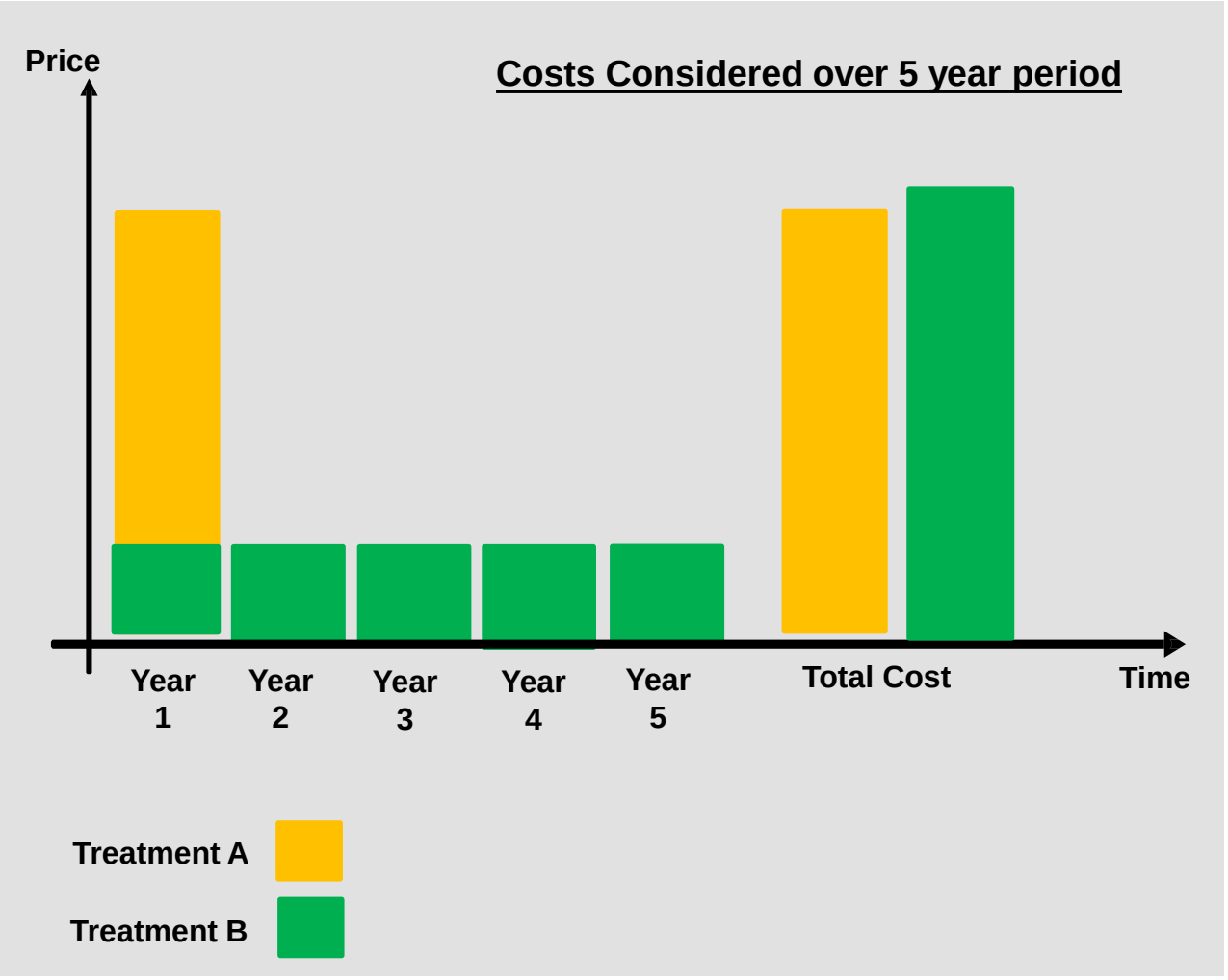
- Cost over what time period? How long does the data suggest the fixed-treatment duration is effective?

**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)

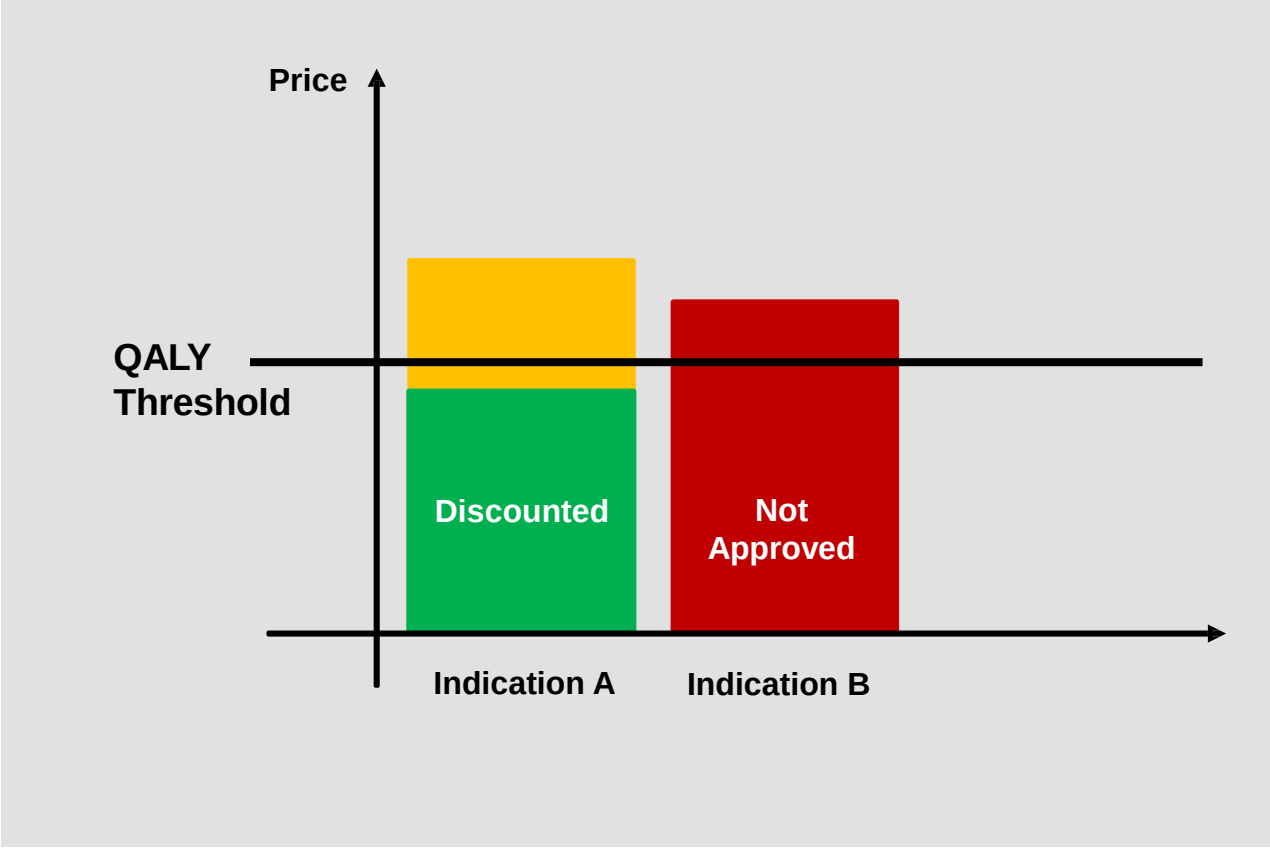
- In reality, whilst most of these address uncertainty in cost-effectiveness, they do little for affordability (unless the price is reduced as a result)

# 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”

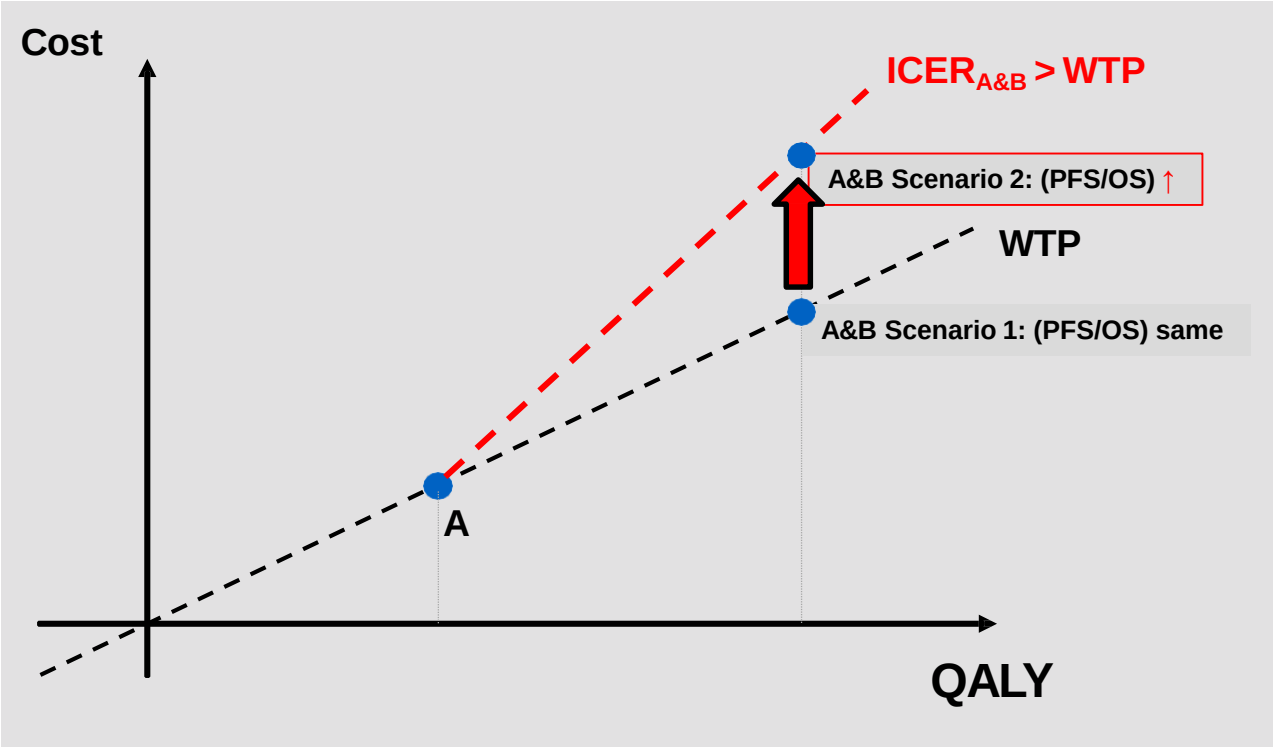



Image from Stefan’s presentation (EXAMPLE 2: THE COMBINATION EXTENDS TIME TO PROGRESSION, BUT WE ASSUME SURVIVAL DATA IS NOT (YET) MATURE

- Agree with SW, 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%

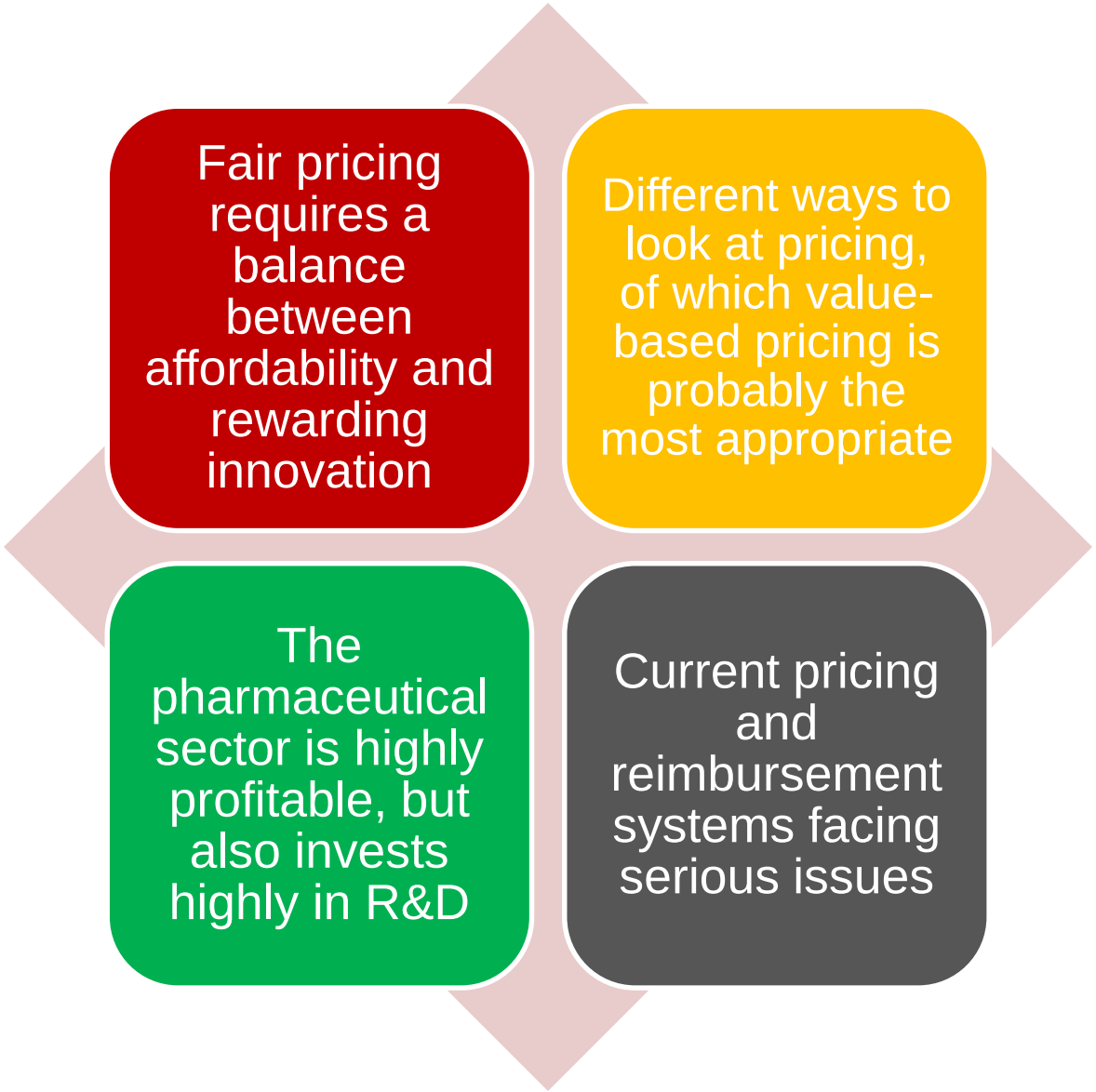
| NICE QALY Thresholds | |
|---|---|
| **NICE Process** | **QALY Threshold** |
| Standard | 20,000 – 30,000 |
| End of Life | 50,000 |

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*

# 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

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