Funding innovation for treatment for rare diseases: adopting a cost-based yardstick approach

Aidan Hollis

Department of Economics

University of Calgary

The cost of new drugs

- The price points for new drugs are increasing rapidly
- Orphan drugs have become a big business and are absorbing an increasing share of total insurer spending, not so much because of volume as because of pricing.

How drugs are normally priced…

- Drug pricing is a mystery.
- But how payers decide whether to accept a given price is not a mystery.
- Usually, payers evaluate the value of the drug in terms of the incremental Quality-Adjusted Life-Years or something similar. If the drug falls below some threshold cost per QALY, they pay for it.

The justification for high prices

- But many orphan drugs have prices that don't meet the value threshold.
- In this case, companies often justify high prices on the basis of small patient populations.
- The cost of development is little changed, but the number of patients is smaller.

Implications of the small population justification

- If payers accept a high price on the basis of high per capita costs of drug development, then they are intrinsically using a cost-based scheme of price regulation.
  - This differs from a pricing scheme in which price is based on value.
- Cost of service regulation is widely used and so it makes sense to learn from that experience in this case.

Standard utility regulation

- Good regulatory practice sets an allowed rate of return to attract capital into the industry. The allowed prices for drugs whose high price is justified by high costs should be based on something similar.
- Good regulatory practice requires an open framework, with evidence tested.

How should cost-based pricing work?

1. A small patient population shouldn't imply that *any* price is OK.
2. The allowed revenues should reflect the ex ante expected cost of development, including adjustment for risk of failure and cost of capital, plus costs of production and distribution etc
   - i.e. the allowed revenues should be based on average costs of drug development for orphan drugs.
   - Then firms have incentives to minimize their development costs

An example for the UK

- Suppose that the average new orphan drug development requires capital costs of $1bn.
- The UK share of OECD GDP is 5%, so the UK's share of paying for a new drug's development costs should be $50m.
- With discounting, this implies that the firm should earn $8m per year over 10 years above the cost of production and distribution.

The orphan premium per patient

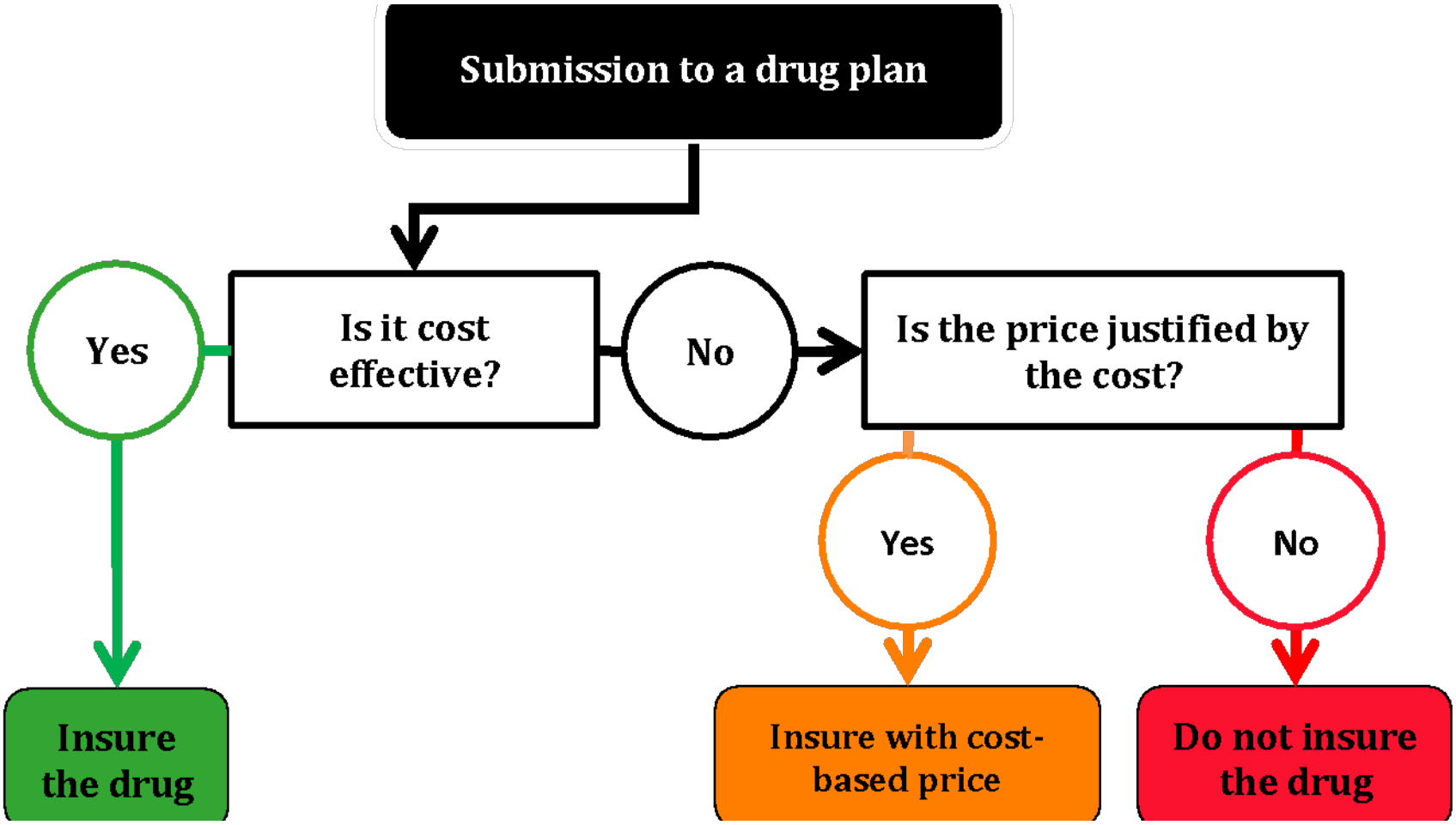
- $8m per year premium implies:
- 600 patients ➔ need to set a price high enough to cover $13,000 per year attributable R&D costs plus costs of production and distribution
  - Kalydeco: 600 patients, actual price per patient per year > $200k
- 200 patients ➔ $40,000 per year attributable R&D costs
  - Soliris: 200 patients, actual price per patient per year >$500k

But suppose my cost estimates are wrong

- Let's suppose that the average, risk-adjusted cost per new orphan drug is $2.5bn, including the cost of capital.
- Then the UK's share of cost should be ~$20m per year over ten years. Attributable R&D cost per patient per year:
  - For Kalydeco $33,000 (price >$200,000)
  - For Soliris $100,000 (price >$500,000)

Cost-based pricing as a tool

- This approach to drug pricing is a tool to establish at least an upper bound on a reasonable price to pay.


Submission to a drug plan
Is it cost effective?
Yes
No
Is the price justified by the cost?
Yes
No
Insure the drug
Insure with cost-based price
Do not insure the drug

Notes

- If the total drug budget is more or less fixed, spending too much on one drug just reduces the amount that can be spent on others.
  - This does not benefit the industry overall
  - And it harms patients
- Prices that far exceed the sum of production, distribution, and R&D costs are not good for patients or the industry.
- If payers have clear rules, that will help industry set appropriate prices.