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## Specific Value Assessment Considerations

### Affordability – When is it a Concern?

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## Affordability within CEA/Value-Based Systems: CEA Threshold and Budget must be Related

- Sustained affordability requires that the Cost-Effectiveness threshold (K) is consistent with the Budget (B)
  - Larger budget => higher K
    - Higher K => more drugs are reimbursed/at higher prices
  - In market-based health systems, K and the budget (premium) are simultaneously chosen, may differ across plans
    - Reflect enrollees Willingness to Pay (WTP) for health care
  - In public health systems, K and B should reflect taxpayer WTP in long run
    - In short run, if B is fixed, K may have to adapt to assure affordability

## Expanding the Measure of Value: Implications for Thresholds, Budgets and Affordability

- Measuring secondary benefits beyond QALYs (e.g. insurance value) is often suggested to support use of a higher threshold K
- But if secondary benefits are measured for drugs, they must be measured for all other goods and services, health and non-health
- Expanding the measure of value could raise or lower K\* (the threshold cost per expanded QALY), if B is fixed
  - All services have more “value” => the cut-off may have to be lower
- Expanding measure of value may not support a higher health Budget
  - Non-health goods (the opportunity cost of health spending) also have secondary benefits

## Specific Affordability Challenges: 1. High Price/High Volume

- High price/high volume drugs can appear unaffordable initially
  - i.e. paying for all potentially eligible patients for a cost-effective new treatment would exceed budget
- Most likely if new drug is:
  - 1) highly effective (“cure”) => high price, and
  - 2) treats chronic, progressive disease => large stock of eligible patients
- Such high price/high volume affordability challenges are often transitory
  - Once accumulated patient stock is treated, annual volume is modest
- Stratified treatment of initial patient stock can spread budget impact

## 2. Very Low Volume/Very High Price: Orphan Drugs

- 1984 Orphan Drug Act (ODA): statutory R&D incentives for orphan drugs
  - R&D tax credits
  - 5 year market exclusivity, etc.
- Informally, orphan drugs get significantly higher prices than non-orphans
  - ROI for orphan drugs now higher than non-orphans (*Evaluate Pharma*)
- ODs raise affordability concerns: ODs now account for 30-40% of NDAs

## Should Volume – Blockbuster or Orphan -- Be Considered in Value Assessment?

- An inverse price/volume relationship cannot be rationalized if price is based on Value
- An inverse price/volume relationship might be rationalized if price is based on Costs, and some costs are fixed (invariant to patient volume)
  - E.g. Drug discovery costs may be quasi-fixed
  - But FDA adapts trial size and other regulatory requirements to reflect patient volume etc. => total R&D costs pre-tax are not fixed
    - Even before ODA's extra tax credits and exclusivities
- Incorporating Volume into Value frameworks lacks theoretical and empirical support, for either blockbuster or orphan drugs

## Conclusions

- Affordability implies a relationship between Budget and Threshold K
- Expanded value measurement for drugs may not justify a higher K
  - Consistency requires expanded value measurement for all goods and services....and resources are fixed
- Unaffordability appears related to volume: blockbusters and orphans
- Unaffordability of high volume/high price drugs is usually transitory
  - Budget impact dissipates, once accumulated patient stock is treated
- Incorporating high/low volume in value assessment for pricing raises unresolved theoretical + empirical issues
  - Should cost assessment be included with value assessment? Why? How?
  - More research required