Perspectives on cost-effectiveness thresholds in the United States

Moderated by:
• Dr. Rick Chapman, Director of Health Economics
Webinar 5: Different thresholds, different perspectives? Equity considerations and the choice between health sector versus societal perspectives

Main Presentation:

Dr. Steven Pearson
President
ICER
Ethical considerations in the selection of cost-effectiveness thresholds for the US
Outline

• Distinctive ethical issues related to having a cost-effectiveness threshold for the US

• Ethical issues related to having one threshold or many: Are there “special cases”?

• Ethical issues related to selection of a base case modeling perspective

• Ethical issues related to the integration of cost-effectiveness thresholds into decision-making in the United States: What is the role of ICER?
Ethical dimensions of not having an ICER for the US

• Loss of health, income, and well-being through the misallocation of pooled, limited resources for health care and other social goods

• Equity concerns
  • Inequality in health and income driven by lack of transparency in variation in care standards across insurers
    • Medicaid versus VA versus private insurers versus Medicare
  • Inequality in health and income driven by lack of transparency to guide voting that determines public policy toward health spending

• Political concerns
  • Corrosive lack of transparency in decision-making drives greed, cynicism, and divisiveness within the health system and beyond
Ethical dimensions of willingness to pay approaches to an ICER for the US

• Equity concerns: whose willingness to pay?
  • Highly variable with income
  • May assume that background labor markets are fair (VSL)
  • If an “average” WTP is selected, people with lower incomes may be forced to pay too much for health care to satisfy the WTP of the rich
  • Societal resources may be drawn into health spending from other domains of social spending much more important to people with lower incomes (e.g. public education)
  • People with lower incomes may be forced out of insurance markets
Ethical dimensions of opportunity cost approaches to an ICER for the US

• Equity concerns
  • Is the current budget for health care normative?
    • In high-income countries with mixed public/private systems, does the budget for publicly funded care provide enough to assure equality of opportunity?
    • In LMIC countries, is the threshold identified by opportunity cost considered normative when instead it should be a call to action?
Selection of a base case perspective for the threshold

• In favor of using the societal perspective in the US
  • Thresholds may reflect effects of health care outside the health system
  • Will push private insurers to value important effects of care outside the health system
  • Captures society’s ethical values related to prioritization of care for younger, working individuals

• In favor of using the health system perspective in the US
  • Private insurers (maybe even Medicaid and Medicare) hold budgets for health care whereas other budgets exist to support desired outcomes outside the health system
  • May overstate or misrepresent society’s ethical values related to prioritization of care for younger, working individuals
  • If opportunity cost is the preferred justification for the threshold, data do not exist to set this threshold looking at costs and effects outside the health care system
One threshold or many: Are there “special cases”?

- Different thresholds for different segments of the population?
- Do past decisions and efficiency frontiers capture social values?
  - “Is” versus “Ought”
- Orphan conditions
  - Social value for rarity?
- End-of-life, severity, lifetime burden of illness, “need”
  - Stepwise ICERs using absolute or proportional QALY shortfall?
- Broader question of quantitative versus qualitative consideration
Fair Price, Fair Access, Future Innovation

Long-Term Value for Money
- Comparative Clinical Effectiveness
- Incremental Cost-Effectiveness
- Other Benefits or Disadvantages
- Contextual Considerations

Short-Term Affordability
- Potential Budget Impact

Fair Price, Fair Access, Future Innovation

Short-Term Affordability
- Potential Budget Impact

Long-Term Value for Money
- Comparative Clinical Effectiveness
- Incremental Cost-Effectiveness
- Other Benefits or Disadvantages
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Integration of thresholds into decision-making: Cost-effectiveness ranges and other considerations

• “Social value judgments”, “Potential other benefits,” and “Contextual considerations”
  • Qualitative integration
  • Quantitative integration
    • Multi-Criteria Decision Analysis

• Budget impact

• Key question: do other considerations push up the ICER or help distinguish value within an ICER range?
Integrating cost-effectiveness thresholds into decision-making in the United States: What is the role of ICER?
### ICER’s Value-based Price Benchmarks (2018-2019)

<table>
<thead>
<tr>
<th>Drug category</th>
<th>Minimum Recommended Discount*</th>
</tr>
</thead>
<tbody>
<tr>
<td>Luxturna for childhood blindness</td>
<td>50%</td>
</tr>
<tr>
<td>Kymriah (CAR-T) for ALL</td>
<td>No discount</td>
</tr>
<tr>
<td>Yescarta (CAR-T) for NHL</td>
<td>No discount</td>
</tr>
<tr>
<td>Hemlibra for hemophilia A</td>
<td>Cost-saving</td>
</tr>
<tr>
<td>Cystic Fibrosis</td>
<td>72%</td>
</tr>
<tr>
<td>CGRPs for migraine prevention</td>
<td>25%</td>
</tr>
<tr>
<td>Elagolix for endometriosis</td>
<td>No discount</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Drug category</th>
<th>Minimum Recommended Discount*</th>
</tr>
</thead>
<tbody>
<tr>
<td>Apalutamide for prostate cancer</td>
<td>No discount</td>
</tr>
<tr>
<td>Psoriasis IL-23s and Cimzia</td>
<td>57%</td>
</tr>
<tr>
<td>Inotersen, patisiran (amyloidosis)</td>
<td>90%</td>
</tr>
<tr>
<td>Hereditary Angioedema</td>
<td>28%</td>
</tr>
<tr>
<td>Eosinophilic asthma biologics</td>
<td>62%</td>
</tr>
<tr>
<td>Spinraza</td>
<td>83%</td>
</tr>
<tr>
<td>Zolgensma</td>
<td>No discount</td>
</tr>
</tbody>
</table>
Use of ICER Assessments: Payers and Providers

• Medicaid programs: New York
  • 2017 law establishing drug spend target
  • If spending ahead of trend allowed to identify drugs for evaluation of value
  • If companies and Medicaid cannot come to agreement on lower price Medicaid can trigger public process to determine specific target price for supplemental rebate
  • 2018 experience and Orkambi
## Orkambi Base Case Results

<table>
<thead>
<tr>
<th>Treatment vs. Best Supportive Care</th>
<th>Cost Per LY Gained</th>
<th>Cost Per QALY Gained</th>
<th>Cost Per Exacerbation Averted</th>
</tr>
</thead>
<tbody>
<tr>
<td>Orkambi</td>
<td>$1,302,766</td>
<td>$891,270</td>
<td>$326,962</td>
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</tbody>
</table>

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### Threshold Price Analysis for Orkambi

<table>
<thead>
<tr>
<th>Annual cost at list price</th>
<th>$50,000 /QALY</th>
<th>$100,000 /QALY</th>
<th>$150,000 /QALY</th>
<th>$200,000 /QALY</th>
<th>$300,000 /QALY</th>
<th>$500,000 /QALY</th>
</tr>
</thead>
<tbody>
<tr>
<td>$272,886</td>
<td>$58,790</td>
<td>$70,991</td>
<td>$83,193</td>
<td>$95,394</td>
<td>$119,797</td>
<td>$168,604</td>
</tr>
</tbody>
</table>

- New York Medicaid DURB deliberation and vote
Use of ICER Assessments: New York Medicaid

Medicaid Is Right to Demand Lower Drug Prices

New York State can’t afford $250,000 a year for one cystic fibrosis medicine.

By Peter B. Bach
May 1, 2018, 7:00 AM EDT
Use of ICER Assessments

• Medicaid programs: Massachusetts joins New York

• VA

• Private payers and PBMs
  • Structural application to health benefit designs: CVS

• Discussion of use for Medicare negotiation/arbitration
Conclusion

• What is ICER’s goal and how does that inform our selection of threshold(s)?

• Do we seek to support differentiated ICERs for different payers or a single threshold range for all payers?
  • Ethical goal tempered by reality

• What is our environment?
  • Cost-increasing search for high-value increased spending in the US health care system
  • Cost-balancing search for most effective use of existing resources
  • Cost-reduction in the US health care system
Life expectancy vs. health expenditure over time (1970-2014)

Health spending measures the consumption of health care goods and services, including personal health care (curative care, rehabilitative care, long-term care, ancillary services and medical goods) and collective services (prevention and public health services as well as health administration), but excluding spending on investments. Shown is total health expenditure (financed by public and private sources).

South Korea spends less than 1/3 of the US per capita
Japan, Australia and France spend less than 1/2 of the US per capita

US life expectancy is far lower than our global competitors.

Health Expenditure (adjusted for inflation and PPP-adjusted for price differences between countries)

Data source: Health expenditure from the OECD; Life expectancy from the World Bank. Licensed under CC-BY-SA by the author Max Moser. The interactive data visualization is available at OurWorldinData.org. There you find the raw data and more visualizations on this topic.
Conclusion

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• If Chuck and Dave get the same answer, and $k \sim v$, what does that suggest?

• No matter what threshold or range we select, we have a responsibility to educate potential users of our work about the need to embed CEA in a broader decision-making structure sensitive to the ethical dimensions inherent in any priority-setting process
Discussion

Responders: Karl Claxton, Patricia Danzon
DIFFERENT PERSPECTIVES AND EQUITY CONSIDERATIONS: ICER USE IN THE US

Patricia Danzon, PhD
Professor Emeritus
The Wharton School
University of Pennsylvania

August 2019
Ethical Dimensions of Not Using (an) ICER(s) in the US

- If payers consistently use an ICER, this not only guides consistent coverage and resource allocation but also constrains prices.
- If payers use ICERs, producers are induced to adopt prices commensurate with incremental value.
  - In order to meet the ICER threshold and get reimbursed.
- Not using ICER thresholds foregoes this economically sound approach to constraining list prices.
  - This has enabled the unconstrained rise in prices in the US.
  - And focused current debate on other, inefficient approaches to price constraint.
Ethical Dimensions: WTP Approaches Need Not Imply Inequity

- Variation in WTP in theory justifies use of different ICERs for different plans in USA
- The WTP of the poor should include not only their personal WTP, which may be very low, but also the altruistic WTP of taxpayers for the poor
  - As in tax support for Medicaid, Medicare, ACA subsidies etc.
- Like many other countries, the US could have a system that guarantees good basic coverage for all, as a floor
  - But allow wealthier people to buy more generous coverage, including use of higher ICERs
- NB: The WTP of wealthier people should be measured net of the tax subsidy to employer contributions
  - This seriously distorts measurement of true WTP of the wealthy
Implementing different ICERs across plans in US

- Theoretically desirable --- but implementation may require confidential rebates
- If plan A uses $100k/QALY and plan B uses $150k/QALY, B would have more drugs covered but would also in principle be willing to pay higher prices for the inframarginal drugs also covered by plan A. If plan A’s prices are visible, plan B will want these lower prices.
  - Differential pricing across plans may require letting producers set list prices based on the highest ICER/WTP + give confidential rebates to plans using lower ICERs.
- Price confidentiality may be essential to enable price differences between plans, as implied by different ICERs
- Better mechanisms may be needed to ensure that rebates given are passed through to consumers/taxpayers who ultimately pay for the health plans and choose the ICERs.
If we use a broad, societal measure for health outcomes, this same breadth must be applied to the services foregone to buy more health

- e.g. education raises wages, improves life expectancy, employability etc.

Current opportunity cost measurement is not yet up to this task

I question the presumption of “society’s ethical values related to prioritization of care for younger, working individuals”

- Youth is captured by potentially more LYS
- “Working” as LFP ignores the real social value of work outside the labor force e.g. home making, care of family etc.
Thank you!

Webinars have been recorded and are all on the ICER website under Commentaries: https://icer-review.org/blog/webinar-series-ce-thresholds/