AMCP Webinar

Cost-effectiveness analysis:
Balancing value with affordability?

Michael Drummond, Dan Danielson and Steven D. Pearson

MODERATOR:
Michael Drummond, PhD

University of York UK
Cost-Effectiveness Analysis: Balancing Value With Affordability?

Michael Drummond
Centre for Health Economics,
University of York

Use of Cost-Effectiveness Analysis Worldwide

• Cost-effectiveness analysis (CEA) is well-established in the formulary decision-making process in many jurisdictions
• These include around half the countries in the European Union, Canada, Australia and several countries in Asia and Latin America
• Typically, these countries have large public payers with the resources to evaluate manufacturer submissions
• Affordability/budget impact is normally assessed
• However, the role of CEA in the US is uncertain
Dymaxium Surveys of US Payers

- Surveyed the 1,200+ US Healthcare Decision Makers registered on the AMCP eDossier system in October 2014, April 2015 and September 2015
- Between 70 and 100 responses to the three surveys
- Asked questions about attitudes towards cost-effectiveness and industry-produced models
- Also, explored the concerns that decision-makers had about the evidence presented to them and the other sources of evidence they consulted

Payers’ Use of Industry Provided Models

How often do they consult industry models:

- Cost-effectiveness Models:
  - Often: 31.76%
  - Sometimes: 65.88%
  - Never: 60%

- Budget Impact Models:
  - Often: 37.65%
  - Sometimes: 60%

2014
Payers’ Use of Industry Provided Models

In what ways do they use industry models?

- To learn about the manufacturer’s clinical data: 91.8%
- To scrutinize the cost-effectiveness analysis: 90.5%
- For background information on the disease: 84.7%
- To help conduct your own analysis: 84.7%
- To repopulate the model with local data: 72.9%
- Often/Sometimes
- Never

What tools and resources do you currently use to assess product affordability? (multiple responses possible)

- Administrative claims data analyses: 52.6%
- Budget impact models and results: 49.5%
- Cost-effectiveness models and results: 48.4%
- Manufacturer dossiers: 48.4%
- Expert opinion: 39.0%
- Actuarial models and results: 22.1%
- Other: 3.2%
SPEAKER:
Dan Danielson, MS, RPH
Pharmacy Manager, Clinical Services, Premera Blue Cross.
Background

Value-Based Insurance Design (VBID)

Concept:
• Align out-of-pocket costs with the value of health services:
  – Different health services have different levels of value
  – Reduce member cost barriers to high-value treatments
  – Discourage low-value treatments by raising out-of-pocket costs

• Expected Result:
  – Improved health outcomes at any level of health care expenditure.
  – Studies show that when barriers are reduced, significant increases in
    patient compliance with recommended treatments and potential cost
    savings result

-Center for Value-Based Insurance Design
-University of Michigan, www.sph.umich.edu/vbidcenter/

Background

VBRx is built using VBID concepts and is unique in the US market

• Guiding Principles
  – Premera’s core values
  – Transparent processes
  – Evidence-based
  – Internal and external decision-making committee
  – Leverage input from practicing physicians and other providers
  – Uses clinical and economic data to determine value

• Academy of Managed Care Pharmacy defines value as:
  – “Value in health care relates to whether a medical intervention...
    improves health outcomes **enough** to justify additional dollars
    spent compared to another intervention.”
Committees

Specialized functions; working in tandem not isolation

- Pharmacy & Therapeutics Committee
  - Clinical evaluation: Safety, effectiveness
  - 7 MDs, 3 pharmacists, 1 lay member – no Premera associates
- Value Assessment Committee
  - 1 MD, PhD, Practicing Internist and Health Economist, Fred Hutchinson Cancer Research Center (chair)
  - 3 PhD Pharmacoeconomist (vice chair)
  - 1 PhD, Bioethics, UW
  - 1 each
    - Community-based oncologist
    - Community-based cardiologist
    - Lay member

How is value measured?

Clinical dimension:

- Incremental Clinical Effectiveness
  - Therapeutic effect size versus
    - Placebo
    - Comparator therapy
    - Cure disease/prolong life or survival (progression free or overall)
  - Adverse effect profile
  - Use/avoidance of use of other medical services
    - Office visits
    - Lab tests
    - Medical procedures
How is value measured?

Human dimension:
• Impacts on patient quality of life
  – Activities of Daily Life
  – Social Role function
    • Spouse/Parent
    • Employment
    • Community
  – Psychological function

Clinical and humanistic outcomes drive the quality of life measure- Quality Added Life Years (QALY)

How is value measured?

Economic dimension ($):
• Incremental Costs versus comparator therapy
  – Costs associated with
    • Drug/procedure costs
    • Treating adverse effects
    • Office visits
    • Lab tests
    • Medical procedures
How is value measured?

Incremental cost effectiveness ratio (ICER)

\[
\text{Incremental cost of therapy (\$)} \over \text{Incremental Quality Added Life Years (QALY)}
\]

- NOT commonly used in USA
- Used EU, Canada and elsewhere
  - Validated surveys of actual target patients (or similar)
  - Some caution using surveys conducted outside of target population/country

More than a calculation

Premera Value Matrix

<table>
<thead>
<tr>
<th>Category</th>
<th>Factor</th>
<th>Evaluation of Relevant Considerations</th>
</tr>
</thead>
<tbody>
<tr>
<td>Clinical Benefit</td>
<td>Research Question</td>
<td>Strength of Evidence</td>
</tr>
<tr>
<td></td>
<td>Safety</td>
<td></td>
</tr>
<tr>
<td></td>
<td>Efficacy</td>
<td></td>
</tr>
<tr>
<td></td>
<td>Effectiveness</td>
<td></td>
</tr>
<tr>
<td>Cost-Effectiveness</td>
<td>Base Case</td>
<td>$20,000-$30,000/QALY</td>
</tr>
<tr>
<td>Analysis</td>
<td>High Estimate</td>
<td></td>
</tr>
<tr>
<td></td>
<td>Low Estimate</td>
<td></td>
</tr>
<tr>
<td>Societal Values</td>
<td>Ethical Issues</td>
<td>Affordability of ___ is essential. While cost effective, the costs of these drugs make widespread coverage impossible within a financially responsible manner. Therefore prioritization of treatment given the very high cost is essential. Prioritization needs to guard against discrimination against patients because others disapprove of the behavior that led to infection (needle sharing, etc.)</td>
</tr>
<tr>
<td></td>
<td>Rare Disease</td>
<td>Yes</td>
</tr>
<tr>
<td></td>
<td>Unmet Need</td>
<td>No</td>
</tr>
<tr>
<td></td>
<td>Other Societal Consideration</td>
<td>Potential for ___ is substantial.</td>
</tr>
<tr>
<td>Regulatory Issues</td>
<td>None noted</td>
<td></td>
</tr>
<tr>
<td>Budget Impact</td>
<td>Pharmacy Budget Impact</td>
<td>PMPM</td>
</tr>
<tr>
<td></td>
<td>Medical Budget Impact</td>
<td>N/A</td>
</tr>
<tr>
<td></td>
<td>Base Case</td>
<td>UX</td>
</tr>
<tr>
<td></td>
<td>High Estimate</td>
<td>XY</td>
</tr>
<tr>
<td></td>
<td>Low Estimate</td>
<td>XZ</td>
</tr>
</tbody>
</table>
Finding economic information
*Credible sources*

• Center for the Evaluation of Value and Risk in Health (CEVR, Tufts University) CEA Registry
• National Institute for Health and Care Excellence (NICE)
• PubMed.gov
• Institute for Clinical and Economic Review
• Manufacturers Models
• Value Assessment Committee Members

U.S. Payers Should Use Models

• **Cost-effectiveness models**
  – Support value-based benefit (VBID) designs
  – Reduce copay access barriers to high value drugs
  – Provide means to evaluate drug price
  – Identify clinical nuances and inappropriate pricing

• **Budget impact**
  – Complements CEA
  – Supports discussion of affordability
  – Disease-based models total costs of care

• **Most of the models we reject are eliminated on clinical grounds, not technical flaws**
What Makes a Good Model?

- **Addresses decision makers’ information needs**
  - What decision are they making?
  - What do they need to inform that decision?
  - Which model type best fits the disease state and setting?

- **Has “Real World” clinical relevance**
  - To clinicians and patients in the plan’s population
  - Reflects actual clinical practice
  - **Models with faulty clinical assumptions will be rejected**

- **Uses Transparent methodology**
  - Per decision makers’ guidance (AMCP/ISPOR)
  - Open, unlocked spreadsheets with good documentation

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What’s missing?

* A measure for health system affordability

Just because a product may have good clinical value does not necessarily mean that it is affordable (budget impact).

*Drugs don’t work in patients who don’t take them*
  
  -C. Everett Koop*
SPEAKER:
Steven D. Pearson, MD, MSc
President and Founder, Institute for Clinical and Economic Review.

Evaluating the Value of New Drugs and Devices
ICER Value Assessment Policy Development Group*

- *NB: All participants provided input into the development of the value assessment framework but none should be assumed to approve of its approach

- Insurers and Pharmacy Benefit Management Companies
  - Aetna
  - Wellpoint
  - Kaiser Permanente
  - OmedaRx
  - Premera
  - America’s Health Insurance Plans (AHIP)

- Patient Organizations
  - FamiliesUSA

- Physician Specialty Societies
  - ASCO

- Manufacturers
  - Merck
  - Covidien
  - Lilly
  - GSK
  - Philips
  - Amgen
  - National Pharmaceutical Council (NPC)
  - Biotechnology Industry Organization (BIO)

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ICER Value Framework 3.0

<table>
<thead>
<tr>
<th>Comparative Clinical Effectiveness</th>
<th>Incremental cost per clinical outcomes achieved</th>
<th>Other benefits or disadvantages</th>
<th>Contextual Considerations</th>
<th>“Care Value” Potential</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>High</td>
<td>Intermediate</td>
<td>Low</td>
<td>High</td>
</tr>
<tr>
<td>“Care Value” Potential</td>
<td>Discuss and voted upon during public meetings</td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Potential Short-Term Health System Budget Impact</th>
<th>Provisional “Health System Value”</th>
<th>Mechanisms to Maximize Health System Value</th>
<th>Achieved “Health System Value”</th>
</tr>
</thead>
<tbody>
<tr>
<td>High Intermediate Low</td>
<td>Discussed and voted upon during public meetings</td>
<td>Discussed during public meetings; included in final ICER reports</td>
<td>Not evaluated by ICER or voted upon by public panels</td>
</tr>
</tbody>
</table>
Incremental Cost per Outcomes Achieved

- Incremental Cost per Outcomes Achieved
  - Long-term perspective
  - Cost per quality-adjusted life year (QALY) gained
    - Associated with high care value
      - <$100,000/QALY
    - Associated with intermediate care value
      - $100-150K/QALY
    - Associated with low care value
      - >$150,000/QALY

A Value Assessment Flowchart
Provisional Health System Value

- Integration of long-term care value with consideration of potential short-term budget impact

- Why short-term budget impact as a part of value?
  - A potential budget impact for an individual drug estimated to contribute significantly to cost growth above some threshold should serve as an "alarm bell" for greater scrutiny and for efforts to maximize health system value

Potential Budget Impact Threshold

- How much potential budget impact is “too much”?  
- Key assumption based on national and state legislation
  - The United States would like to take measures so that overall health care cost growth does not outstrip growth in the national economy

- Measure
  - The amount of net cost increase per individual new intervention that would contribute to growth in overall health care spending greater than the anticipated growth in national GDP + 1%
### Summary of Potential Budget Impact Threshold Calculations

<table>
<thead>
<tr>
<th>Item</th>
<th>Parameter</th>
<th>Estimate (Drugs)</th>
<th>Estimate (Devices)</th>
<th>Source</th>
</tr>
</thead>
<tbody>
<tr>
<td>1</td>
<td>Growth in US GDP, 2015-2016 (est.) +1%</td>
<td>3.75%</td>
<td>3.75%</td>
<td>World Bank, 2015</td>
</tr>
<tr>
<td>2</td>
<td>Total health care spending ($)</td>
<td>$3.08 trillion</td>
<td>$3.08 trillion</td>
<td>CMS NHE, 2014</td>
</tr>
<tr>
<td>3</td>
<td>Contribution of drug/device spending to total health care spending (%)</td>
<td>13.3%</td>
<td>6.0%</td>
<td>CMS NHE, Altarum Institute, 2014</td>
</tr>
<tr>
<td>4</td>
<td>Contribution of drug spending to total health care spending (%)</td>
<td>$410 billion</td>
<td>$185 billion</td>
<td>Calculation</td>
</tr>
<tr>
<td>5</td>
<td>Annual threshold for net health care cost growth for all new drugs (Row 1 x Row 4)</td>
<td>$15.4 billion</td>
<td>$6.9 billion</td>
<td>Calculation</td>
</tr>
<tr>
<td>6</td>
<td>Average annual number of new molecular entity or device approvals, 2013-2014</td>
<td>34</td>
<td>23</td>
<td>FDA, 2014</td>
</tr>
<tr>
<td>7</td>
<td>Annual threshold for average cost growth per individual new molecular entity (Row 5 x Row 6)</td>
<td>$452 million</td>
<td>$301 million</td>
<td>Calculation</td>
</tr>
<tr>
<td>8</td>
<td>Annual threshold for estimated potential budget impact for each individual new molecular entity (doubling of Row 7)</td>
<td>$904 million</td>
<td>$603 million</td>
<td>Calculation</td>
</tr>
</tbody>
</table>

### From Value Assessment to “Value-Based Price Benchmarks”

<table>
<thead>
<tr>
<th>PCSK9 Drugs</th>
<th>Care Value Price: $100K/QALY</th>
<th>Care Value Price: $150K/QALY</th>
<th>Max Price at Potential Budget Impact Threshold</th>
<th>Draft Value-Based Price Benchmark</th>
</tr>
</thead>
<tbody>
<tr>
<td>List price $14,350 (n=2,636,179)</td>
<td>$5,404</td>
<td>$7,735</td>
<td>$2,177</td>
<td>$2,177</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Entresto</th>
<th>Price to Achieve $100K/QALY</th>
<th>Price to Achieve $150K/QALY</th>
<th>Max Price at Potential Budget Impact Threshold</th>
<th>Draft Value-Based Price Benchmark</th>
</tr>
</thead>
<tbody>
<tr>
<td>List price $4,560 (n=1,949,400)</td>
<td>$9,480</td>
<td>$14,472</td>
<td>$3,779</td>
<td>$3,779</td>
</tr>
</tbody>
</table>
ICER Drug Assessment Expansion

- Support from the Laura and John Arnold Foundation (LJAF)
- Ramping up to produce 15-20 reports per year on highest impact new drugs near time of FDA approval
- All reports to be debated in public by independent committees
- Work with patient, manufacturer, payer, provider, and policymaker communities to enhance uptake and application of reports

Advancing value assessment and pricing for new drugs

- For payers
  - Track and use ICER reports to support value-based coverage decisions and benefit designs
    - Make independent value reports an explicit and transparent part of coverage and price negotiation
    - Apply reports to justify non-coverage, step therapy, or other restrictions if improved comparative clinical effectiveness is not demonstrated
    - If price meets a price benchmark
      - Drug gets first tier and low or no co-pay
      - Drug is “gold carded” with provider groups
    - If price does not meet the benchmark
      - Automatic third tier
      - “Reference price” to value benchmark: Additional costs paid by patients or manufacturers
      - High prior authorization requirements for providers
Thank you

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Questions?
For more information please contact:

Jackie Gladman

jgladman@dymaxium.com or info@dymaxium.com

Stay Tuned

Webinar will be posted on the AMCP website.