Applying Real-world Data in Health Technology Assessment (HTA)
Why One Size Does Not Fit All

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Disclaimer and Acknowledgements

The views expressed in this presentation are those of the presenter, not necessarily those of Xcenda.

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Overview of Health Technology Assessment (HTA)
What is HTA?

Health Technology Assessment

A form of policy research that examines short- and long-term consequences of the application of a healthcare technology¹

Properties assessed include: evidence of safety, efficacy, patient-reported outcomes, real world effectiveness, cost and cost-effectiveness²

Multidisciplinary process that summarizes information about the medical, social, economic and ethical issues related to the use of a health technology in a systematic, transparent, unbiased, robust manner¹

What is the role of HTA?

**Evidence-based Medicine (EBM)**
- EBM is an evidence synthesis and decision process used to assist patients’ and/or physicians’ decisions.
- It considers evidence regarding the effectiveness of interventions and patients’ values and is mainly concerned with individual patients’ decisions, but is also useful for developing clinical guidelines as they pertain to individual patients.

**Comparative Effectiveness Research (CER)**
- CER includes both evidence generation and evidence synthesis.
- It is concerned with the comparative assessment of interventions in routine practice settings.
- The outputs of CER activities are useful for clinical guideline development, evidence-based medicine, and the broader social and economic assessment of health technologies (i.e., HTA).

**Health Technology Assessment (HTA)**
- HTA is method of evidence synthesis that considers evidence regarding clinical effectiveness, safety, cost-effectiveness, and, when broadly applied, includes social, ethical, and legal aspects of the use of health technologies.
- A major use of HTAs is in informing reimbursement and coverage decisions, in which case HTAs should include benefit-harm assessment and economic evaluation.

Confusion Exists Concerning Appropriate Definitions of CER, HTA, and EBM

<table>
<thead>
<tr>
<th></th>
<th>Can it Work? (Efficacy)</th>
<th>Does it Work? (Effectiveness)</th>
<th>Is it Worth It? (Value)</th>
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<tr>
<td>Evidence Generation</td>
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<td>CER</td>
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<td>Evidence Synthesis</td>
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<td>Decision-Making</td>
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Redefined Relationships of Evidence Processes

<table>
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<tr>
<th>Evidence Generation</th>
<th>Evidence Synthesis</th>
<th>Decision Making</th>
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<td>RCT</td>
<td>PCT</td>
<td>Observational Studies</td>
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<td>SRT</td>
<td>SRE</td>
<td>CER</td>
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<td>Clinical Guidelines</td>
<td>Economic Evaluation</td>
<td>Budget Impact</td>
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<td>EBM</td>
<td>Physician &amp; Patient Decision</td>
<td>Coverage Reimbursement Decision</td>
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Solid lines indicate clear relationships, and dotted lines indicate disputed relationships. Diamonds represent decision processes, and circles and ovals represent all other evidence activities, except for the rectangles, which are reserved for EMB, HTA, and CER.
Why HTA?

Informed decision making in healthcare, main purpose¹

Includes decisions made at the patient level, healthcare provider level, up to the national level²

Address the impact of the intervention including direct and indirect consequences²

Inform the formulation of safe, effective, health policies that are patient focused and seek to achieve best value¹

Directly-related to evidence-based medicine²


HTA Stages

Four Stages

Clinical evidence
• Systematic evaluation of evidence for a technology and a requirement of good evidence for such things as coverage, placement on formularies, and affirmative guidelines

Estimate of clinical outcomes
• Benefits and risks
• Benefit-risk ratio

Compare costs and cost-effectiveness

Ethical and legal implications

Source: http://www.inahta.org/upload/HTA_resources/AboutHTA_Resources_for_HTA.
### Who Uses HTA?

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<tr>
<th>Different healthcare decision makers</th>
<th>Regulatory agencies</th>
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<tbody>
<tr>
<td></td>
<td>Healthcare payers</td>
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<tr>
<td></td>
<td>Clinicians and patients</td>
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<td></td>
<td>Hospitals and clinics</td>
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<td></td>
<td>Healthcare product companies</td>
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<td>Managed care organizations</td>
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<td>Government and private sector payers</td>
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Trends in HTA
How good are we at being predictive?
Who would have Predicted?
Berlin, November 1989
Some Relevant HTA Trends

- Increased pressure by countries to restrict access to certain, especially high-price drugs and interventions (value-based assessments)
- Use of Quality of Life / Patient Reported Outcomes (PROs)
- Sub-groups, in part due to the increasing presence of “personalized medicine”
- Use of meta-analysis / indirect treatment comparisons
- Converging of regulatory and HTA/reimbursement bodies
Applying Real-World Data in HTA
“Real World Data” are observations of effects based on what happens after a prescriptive (treatment) decision is made where the researcher does not or cannot control who gets what treatment and does not or cannot control the medical management of the patient beyond observing outcomes

– ISPOR task force
Types of Real-world Data Sources

- Surveys
- Administrative/Claims data
- Electronic health/medical records
- Hospital discharge data
- Patient/drug registries
Show me the evidence!!
Products are under continuous scrutiny across the lifecycle

We are moving from a “launch package” of data to an ever-expanding “lifecycle data file”
Historically, manufacturers were almost the exclusive custodians of data related to their products.
“Real world evidence” even further unveils the value of a product undemonstrated in a clinical trial setting.
Data Options for HTA

Existing Clinical Studies

- Easy, cost-effective
- Demonstrated to be successful
- Provide value for inconclusive studies

Possible overstated results
Do not allow for direct comparisons

Existing Databases

- New, low-cost information
- Real-world data representing usual care
- Potential for enhancement with EMRs

Lack detailed clinical variables
Potential for selection bias

New Clinical Studies

- Greatest potential
- Control for selection bias

Highest cost
Possible ethical concerns
Hopefully the intention is that this is NOT the Case!
What is the Reality of Real World Data?

- Disparate databases often not linkable to each other
  - BUT you can confirm findings using databases from different vendors & build a comprehensive story across database types

- Represent secondary uses of the data for which they were not necessarily collected
  - BUT often have information not thought to collect or available in prospective data collection
  - Relevance of the data set for a country may not be recognized by another one

- Despite all the merits of observational, real world research it is biased
  - BUT there are statistical methods to overcome some biases
How Can Real-World Data be Applied?

- Provide input into Phase III, and possibly Phase II, studies to include relevant endpoints for HTA bodies
- Fill gaps and inform inputs in health economic models
- Obtain a better understanding of product use and current treatment/switching patterns
- Systematic means to help establish the value of a product, and measure real-world outcomes in support of “value-based” assessment/pricing
Concluding Remarks
Implications and Applications
In Conclusion

- Ultimate success in the market place is driven by the relevance & strength of clinical & economic evidence provided to payers & HTA bodies

- Applying real-world evidence properly can provide additional persuasive clinical evidence, especially when evaluating a product against standard of care or competitor product(s)

- Payers’ clinical & economic evidence needs should be considered in constructing the clinical development plan well before Phase III

- However, since differing HTA bodies can still be expected to view the same data differently, one size will simply not fit all
Thank you

Comments, Critiques & Questions

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Where knowledge, reach and partnership shape healthcare delivery.