An Introduction to Health Technology Assessment in the U.S. and Canada: Possible Lessons and Implications for Taiwan?

Presentation to:
Workshop on HTA Policy Formation and Implementation
National Yang-Ming University Performance Center
Sponsor: Taiwan Food and Drug Administration
Organizer: Research Center of Health and Welfare Policy, National Yang-Ming University
Co-Organizer: Center for Drug Evaluation

July 5, 2011
Lou Garrison, Ph.D.
Professor, Department of Pharmacy
University of Washington
Acknowledgements

• Research Collaborators:
  – Adrian Towse and Nancy Devlin, Office of Health Economics, UK
  – Sean Sullivan and David Veenstra, University of Washington
Country Populations and Incomes (IMF/$PPP 2010)

- Taiwan—23 million; $35,227
- Australia—23 million; $39,699
- Canada—34 million; $39,057
- South Korea—49 million; $29,836
- UK—62 million; $34,920
- US—311 million: $47,284
  - California: 37 million
  - Medicare (elderly and disabled): 38 million
  - Uninsured: 51 million
Agenda

• Introduction to Health Technology Assessment (HTA)

• HTA in the U.S. Health Care System

• HTA in Canadian Health Care System

• Principles of HTA

• Study of HTA in Emerging Markets
Key Messages about Health Technology Assessment (HTA)

• **Evolution**—HTA is not new, but it has nearly 30 years of history—an evolutionary one.

• **Globalization**—HTA is being applied in more and more countries, and the number of competent practitioners is growing--globally.

• **Variety**—How HTA is used varies markedly, but depends on incentives to use the information.

• **Challenge**—HTA operates in the political sphere and its role and performance in any given country will depend on how it is institutionalized and organized.
What is HTA? Some Definitions

• Technology assessment is a form of policy research that examines short- and long-term social consequences (for example, societal, economic, ethical, legal) of the application of technology. The goal of technology assessment is to provide policy-makers with information on policy alternatives (Banta 1993).

• Health technology assessment ... is a structured analysis of a health technology, a set of related technologies, or a technology-related issue that is performed for the purpose of providing input to a policy decision (U.S. Congress, Office of Technology Assessment 1994).
Health Technology Assessment (HTA) – Key Processes/Functions

- **Scoping** – Identifying and monitoring pre-approval products that will require assessment and appraisal.

- **Topic Selection** – Post-approval, prioritizing products for assessment and appraisal

- **Assessment** – Mfrs submit evidence, organization undertakes systematic assessment of the evidence (clinical, economic, budgetary)

- **Appraisal** – Decision-making committee considers evidence from assessment, input from stakeholders and makes a recommendation for funding/implementation

- **Funding/Implementation** – Decision by the budget holder to reimburse/cover a product with or without parameters.
Public Sector: Some Do First 3

- **Scoping** – Identifying and monitoring pre-approval products that will require assessment and appraisal.

- **Topic Selection** – Post-approval, prioritizing products for assessment and appraisal

- **Assessment** – Mfrs submit evidence, organization undertakes systematic assessment of the evidence (clinical, economic, budgetary)

- **Appraisal** – Decision-making committee considers evidence from assessment, input from stakeholders and makes a recommendation for funding/implementation

- **Funding/Implementation** – Decision by the budget holder to reimburse/cover a product with or without parameters.
Public Sector: Some Do First 4

- **Scoping** – Identifying and monitoring pre-approval products that will require assessment and appraisal.

- **Topic Selection** – Post-approval, prioritizing products for assessment and appraisal

- **Assessment** – Mfrs submit evidence, organization undertakes systematic assessment of the evidence (clinical, economic, budgetary)

- **Appraisal** – Decision-making committee considers evidence from assessment, input from stakeholders and makes a recommendation for funding/implementation

- **Funding/Implementation** – Decision by the budget holder to reimburse/cover a product with or without parameters.
Private Sector: Does All Steps

- **Scoping** – Identifying and monitoring pre-approval products that will require assessment and appraisal.

- **Topic Selection** – Post-approval, prioritizing products for assessment and appraisal.

- **Assessment** – Mfrs submit evidence, organization undertakes systematic assessment of the evidence (clinical, economic, budgetary).

- **Appraisal** – Decision-making committee considers evidence from assessment, input from stakeholders and makes a recommendation for funding/implementation.

- **Funding/Implementation** – Decision by the budget holder to reimburse/cover a product with or without parameters.
Health Technology Assessment: Principle vs. Practice

• In principle, not just about pharmaceuticals
  – In practice, drugs have been the focus

• In principle, not just about costs
  – In practice, it has been about costs and cost-effectiveness (and budget impact)

• In principle, it’s a scientific approach to resource allocation
  – In practice, it’s often about politics.
Another Separation of HTA Functions

1. **Marketing approval ("Registration")**—usually for drugs and devices, an assessment of benefit-risk balance based on clinical trial or other data

2. **Coverage**—inclusion as a covered service in health plan benefit package

3. **Reimbursement**—establishes plan payment level, perhaps considering “value of money” or budget impact, or via internal (therapeutic) reference pricing or via external (international reference pricing)

4. **Clinical guidelines**—use HTA information use to support clinical guidelines in disease areas.
HTA as an Economic Production Process and Economic Good

• The process of HTA can usefully be thought of as a “technology” or production process.
  – As such, one can ask, whether it is “technically” efficient, obtaining maximum output given the resources used.

• The output of the process is “information”—a “public” good, in economic jargon. Indeed—a global public good
  – One can also ask whether the the production is “economically” efficient, i.e., is it technically efficient, being produced a minimum cost, and in the right quantity?

• Public goods create incentive to be a “free-rider”

Economics says: “public goods” will be undersupplied by private markets. Incentives like patents and subsidies are needed.
Agenda

• Introduction to Health Technology Assessment (HTA)

• HTA in the U.S. Health Care System

• HTA in Canadian Health Care System

• Principles of HTA

• Study of HTA in Emerging Markets
## U.S. Insured Distribution, 2011

<table>
<thead>
<tr>
<th>United States</th>
<th>Number</th>
<th>0 - 150,026,000</th>
</tr>
</thead>
<tbody>
<tr>
<td>Employer</td>
<td>150,026,000</td>
<td></td>
</tr>
<tr>
<td>Individual</td>
<td>14,000,900</td>
<td></td>
</tr>
<tr>
<td>Medicaid</td>
<td>47,375,300</td>
<td></td>
</tr>
<tr>
<td>Medicare</td>
<td>37,583,300</td>
<td></td>
</tr>
<tr>
<td>Other Public</td>
<td>3,683,500</td>
<td></td>
</tr>
<tr>
<td>Uninsured</td>
<td>50,674,300</td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>United States</th>
<th>Percent</th>
<th>0% - 100%</th>
</tr>
</thead>
<tbody>
<tr>
<td>Employer</td>
<td>49%</td>
<td></td>
</tr>
<tr>
<td>Individual</td>
<td>5%</td>
<td></td>
</tr>
<tr>
<td>Medicaid</td>
<td>16%</td>
<td></td>
</tr>
<tr>
<td>Medicare</td>
<td>12%</td>
<td></td>
</tr>
<tr>
<td>Other Public</td>
<td>1%</td>
<td></td>
</tr>
<tr>
<td>Uninsured</td>
<td>17%</td>
<td></td>
</tr>
</tbody>
</table>

Source: Kaiser FF.org
Distribution of National Health Expenditures, by Type of Service, 2009

Note: Other Personal Health Care includes, for example, dental and other professional health services, durable medical equipment, etc. Other Health Spending includes, for example, administration and net cost of private health insurance, public health activity, research, and structures and equipment, etc.


Dollars in Billions:

<table>
<thead>
<tr>
<th>Year</th>
<th>NHE as a Share of GDP</th>
</tr>
</thead>
<tbody>
<tr>
<td>1960</td>
<td>5.2%</td>
</tr>
<tr>
<td>1970</td>
<td>7.2%</td>
</tr>
<tr>
<td>1980</td>
<td>9.2%</td>
</tr>
<tr>
<td>1990</td>
<td>12.5%</td>
</tr>
<tr>
<td>2000</td>
<td>13.8%</td>
</tr>
<tr>
<td>2001</td>
<td>14.5%</td>
</tr>
<tr>
<td>2002</td>
<td>15.4%</td>
</tr>
<tr>
<td>2003</td>
<td>15.9%</td>
</tr>
<tr>
<td>2004</td>
<td>16.0%</td>
</tr>
<tr>
<td>2005</td>
<td>16.0%</td>
</tr>
<tr>
<td>2006</td>
<td>16.1%</td>
</tr>
<tr>
<td>2007</td>
<td>16.2%</td>
</tr>
<tr>
<td>2008</td>
<td>16.6%</td>
</tr>
<tr>
<td>2009</td>
<td>17.6%</td>
</tr>
</tbody>
</table>

U. S. Health Care System

- No explicit or constitutional “right to health care”
- No universal coverage
- Insurance coverage is a complicated public/private mix, with a “safety net” that fails too often.
- Described as: “pluralistic”, “fragmented”, “disorganized,” “inequitable”, “innovative”, “decentralized”...
- No national basic or explicit minimum health care benefit package. (Hence, no need for one national HTA body.)
- Food and Drug Administration review is one form of partial, national HTA
- But plans need not cover FDA-approved products
- And plans can cover some products “off-label”
Two Poles on HTA Continuum

**US:**
Decentralized; Little reliance on formal HTA

**UK:**
Centralized; Heavy reliance on formal HTA
Employer-sponsored health insurance market segments

- Fully or partially insured (approx 150 mill with self-insured)
  - Traditional health insurance plans (e.g. United Healthcare, WellPoint, Aetna,) and closed panel HMOs (e.g. Kaiser, GHC)
  - Have internal/robust HTA programs
- Self-insured companies – With or without administrative services contracts with health plans and/or PBMs (e.g. GM, Boeing)
  - Use HTA programs of the ASO health plan.
- Individual insurance policy – (approx. 14 million)
  - Use HTA programs of the health plan
Government-sponsored (CMS) health care segments

- Medicare (Ages 65+) – (approx. 40 million)
  - MCAC for Part B and medical technology
  - Private health plans for Part D
- Medicaid – (poor and disabled) – (approx. 50 million)
  - State run HTA programs
  - Some supported (assessment) by DERP
- Uninsured – (approx 50 million)
  - 50% pay cash for drug prescriptions
Key U.S. Trends

• Still limited use of CEA in formulary and non-formulary decisions
  – Why? Lack of cost controls in US insurance system

• Growing interest in:
  – “comparative effectiveness research”
  – “real-world” data
  – “coverage with evidence development”
HTA in the US

• Decisions regarding product licensing (FDA) and federal coverage and reimbursement (CMS) are separate.

• Because the US health care system is largely decentralized and privatized, the HTA processes are decentralized and privatized.
  – Adoption, coverage and reimbursement of medical technology is left to the local payers.
HTA in the US - Federal

• Medicare HTA –

  – For non-drug technologies, the Medicare program (Coverage Division) uses the following process:

    • Scopes technologies based on various factors (cost, impact to Medicare, visibility, safety) and then selects technologies.

    • Writes specific questions that determine the scope of the HTA and the search for evidence.

    • Commissions an HTA through a government research organization (AHRQ). AHRQ contracts with an Evidence-Based Practice Center (private vendors and Universities) for the assessment.
HTA in the US - Federal

- Medicare HTA –
  
  For non-drug technologies, the Medicare program (Coverage Division) uses the following process:

  - The Medicare Coverage Advisory Committee (MCAC) receives the document, participates in an open hearing and then votes on the STRENGTH of the evidence, but does not make a decision or give a recommendation to Medicare.

  - The Coverage Division within Medicare makes the final coverage decision weighing the evidence and considering the deliberations from the MCAC.
HTA and CEA - Federal

• What is the role of economic evaluations in Medicare program?
  – By Congress – Medicare is prohibited from using CEA data to *make* coverage and reimbursement decisions, but not from informing decisions.
  – Practically – Employees of CMS do review publicly available CEA and budgetary impact data *to inform* coverage and reimbursement decisions. They do not request these data from manufacturers.
CMS/Medicare national coverage decisions (NCDs)

• Reserved for technologies affecting large number of beneficiaries with greatest impact on Medicare

• Inconsistent local coverage policies

• Technology represents significant potential medical advance

• Technology subject to substantial controversy

• Potential for rapid diffusion or overuse exists

• Formal external request

http://www.medpac.gov/publications/congressional_reports/Mar03_AppB.pdf/
MEDICARE NATIONAL COVERAGE PROCESS

- Preliminary Discussions
  - Benefit Category
    - National Coverage Request
      - Staff Review
        - Draft Decision Memorandum Posted
          - Public Comments
            - Final Decision Memorandum and Implementation Instructions
              - Department Appeals Board

- 6 months
- 30 days
- 60 days

- External Technology Assessment
  - Medicare Coverage Advisory Committee
    - Staff Review
      - 9 months

http://www.cms.hhs.gov/coverage/
CMS national coverage decisions

- NCDs
- Local Coverage Decisions
- No formal decision making process
Medicare (CMS) - Federal

- **Coverage with Evidence Development (CED)**
  - CMS grants *provisional* reimbursement of a technology during which time the manufacturer/developer undertakes a study that would address specific evidence gaps. In some cases, the agencies (FDA, CMS, NIH) would be involved in the trial.
  - Purpose – Allowing limited use/reimbursement of the technology during evidence generation.
Exhibit A. Enhanced formulary review process for biotech drugs, utilizing AMCP Format submissions.

Watkins, Sullivan Health Affairs 2006
HTA Appraisal Process in US Health Plans

New Technology Product

Is it clinically effective?
- Yes
- No

Is it safe?
- Yes
- No

Does it offer improved value?
- Yes
- No

Are there other therapeutically comparable products?
- Yes
- No

Preferred

Nonpreferred

Prefer most cost-effective
Private Sector HTA Trends

• Plan consolidation in the private market (6 plans = >65% of employer sponsored segment).
  – Economies will allow organizations to build rigorous HTA programs.
  – 2009 Implementation of Wellpoint guidelines is very similar to NICE guidance

• Health plans are routinely scanning the HTA reports of ex-US decision making bodies.

• Non-drug technologies and companion diagnostic/drug combinations are now being reviewed as part of the P/T process in some plans

• Budgetary and financial impact is quite important. These estimates should include
  – Pharmacy costs and medical cost offsets
US Private Sector Guidelines—Academy of Managed Care Pharmacy (AMCP)
Academy of Managed Care Pharmacy (AMCP) Policy on Economic Data for Formulary Decision Making

The AMCP guidelines provide a standardized template for a broad unsolicited request for all product-related information, some of which are not currently available to health plans.
AMCP Dossier Components

- Clinical Studies
- Published Economic Evaluations
- Safety
- Efficacy
- Effectiveness
- Disease and Product Description
- Resource Utilization
- Costs
- Plan Data
- Economic Modeling
- Reporting Potential for Bias
- Overall Product Cost and Value
- Submission Document

Value Modeling

Published Economic Evaluations

Resource Utilization

Plan Data

Economic Modeling

Overall Product Cost and Value

Submission Document
Health Reform and CER: Patient-Centered Outcomes Research Institute (PCORI)

Responsibilities:

• Setting priorities
• Developing methodological standards
• Communicating research results to decision makers

• Not allowed to consider cost-effectiveness!
What is Comparative Effectiveness Research (CER)?

- Compares two or more alternative interventions
- Focuses on effectiveness, or real-world outcomes, as opposed to efficacy, or experimental outcomes
- Aims to provide information to a wide range of decision-makers, including patients, providers, and policy makers.
Comparative Effectiveness Research in the Decision Maker-Evidence-Policy Loop

Decision-Makers (with uncertainties):
- Patients [A]
- Providers [B]
- Guideline developers [C]
- Regulators [D]
- Purchasers [E]
- Policymakers [F]

Their Policy Questions:
- Utilization [A & B]
- Medical guidelines [B & C]
- Marketing authorization [D]
- Truth-in-advertising [D]
- Prospective observational
- Retrospective observational
- Qualitative

Comparative Effectiveness Research

Study Design Methodologies
- Experimental
- Quasi-experimental
- Prospective
- Systematic reviews
- Statistical methods
- Models

Data Analysis Methodologies
- Systematic reviews
- Statistical methods
- Models

Evidence

Communication and Implementation of:
- Decisions
- Recommendations

Apply Evidentiary Standards to Reach
Decisions about:
- Utilization [A & B]
- Medical guidelines [B & C]
- Marketing authorization [D]
- Truth-in-advertising [D]
- Insurance coverage [E]
- Reimbursement [E]
- Government policies [F]
- Need for more data and evidence [A → F]
Question: The US Pharmacoeconomics Puzzle

- The US has many skilled professionals in pharmacoeconomics, years of production on Health Economics (HE), and the financial resources needed for establishing an HTA. However, the use of HE by payers for decision making is low.

- What are the reasons for this?
- What are the future consequences?

- American healthcare is decentralized in organization and financing.
- Value is of concern to decision-makers, but its use is not explicit.
- Debates focus on the needs for comparative evidence and cost-sharing.
- Economic evidence of value may influence practice guidelines.
- Dramatic shift toward use of CEA “seems unlikely.”
- Change is likely to be incremental and use will remain indirect—unless there is a major political shift.
An Incentives Interpretation

The U.S. reality is not surprising given the incentives:

1. The primacy of employer-based insurance which receives a tax-subsidy:
   - Patients are not very price-sensitive in their insurance (i.e., coverage) choices. (A kind of aggregate moral hazard.)
   - Inequitable.
   - Drives the standard of care in system.

2. Elderly are “grandfathered” into the system.

3. Care is rationed to the uninsured, who can usually obtain access the system through the emergency room.
   - They are delayed in their receipt of the standard of care.
Implications of U.S. incentives (1)

- Incentive is to use care as long as Marginal Health Benefit \( > 0 \).
  \( \rightarrow \) “Flat-of-the-curve” medicine

Payers have limited incentive to make hard choices: they want to keep physicians and patients happy.
Implications of U.S. incentives (2)

• Rationing will remain implicit.  
  → Worse outcomes for those with access problems

→ CEA will play only a very limited role—indirectly in practice guidelines.

→ Scientific standards calling for CEA from a “societal perspective” can create a norm or standard that influences decision-makers.

→ Spending will continue to increase, with growing uninsured population.
Modest (to date) HTA Reforms/Developments in the US

- Coordination of evidence development advice to manufacturers between FDA/CMS
  - Early engagement by mfrs (Novartis)
- BCBSA initiative to coordinate the HTA assessment function of member plans
- CER: Broader role of government in generating comparative evidence of technologies
- State health care authorities establishing robust processes for HTA – Washington State
Things must change!

Spending on Health Care as a Percentage of Gross Domestic Product Under an Assumption That Excess Cost Growth Continues at Historical Averages

Source: CBO, Nov. 2007
Agenda

- Introduction to Health Technology Assessment (HTA)
- HTA in the U.S. Health Care System
- HTA in Canadian Health Care System
- Principles of HTA
- Study of HTA in Emerging Markets
Health Canada: Canadian Food and Drug Administration
Health Technology Assessment: Canada

Mike Tierney (CADTH) presentation on May 4, 2008 at the PhRMA-ISPOR Symposium, entitled “Evolving Evidence Requirements from a payer’s perspective: Canada”
http://cadth.ca/index.php/en/home
Canadian healthcare – 18 health plan system

- Northwest Territories
- Yukon Territory
- Nunavat
- Labrador and Newfoundland
- British Columbia
- Alberta
- Saskatchewan
- Manitoba
- Ontario
- Quebec
- Prince Edward Island
- Nova Scotia
- New Brunswick
Canadian Agency for Drugs and Technologies in Health (CADTH)

- Independent, incorporated, not-for-profit agency
- Founded in 1989
- Funded by the Canadian federal, provincial, and territorial (F/P/T) governments
  - “We need a more coordinated approach across the country to ensure that all Canadians are benefiting from the advances being made in health technology” (Perrin Beatty, Minister of National Health and Welfare, 1989)
Principal stakeholders in health technology management

- **Provinces/territories** (responsible for health care system delivery)
- **Health authorities/hospitals** (delegated purchasing decisions)
- **Health care professionals** (health care delivery)
- **Industry** (designer, tester, manufacturer, information provider)
- **Federal Government** (regulators)
- **Public** (recipient, user, purchaser)
How does CADTH meet these needs?

CADTH's three core programs

- HTA (Health Technology Assessment)
- CDR (Common Drug Review)
- COMPUS (Canadian Optimal Medication Prescribing and Utilization Service)
CADTH's Common Drug Review (CDR)

- A single process for
  - conducting objective, rigorous reviews of the clinical and economic evidence for drugs
  - providing formulary listing recommendations to the publicly funded drug plans in Canada (except Quebec)

- Formulary decisions are made by the drugs plans
  - based on CDR recommendation, and plan mandates, priorities and resources

- Objectives
  - reduce duplication, maximize use of limited resource and expertise, provide equal access to evidence and advise
Drug assessment – CDR's role

FEDERAL GOVERNMENT
Approval of new drugs for sale in Canada

CADTH's CDR Program
Evidence-based reviews
Common listing recommendations (CEDTH)

F/P/T DRUG PLANS
Listing/coverage decisions
Common Drug Review (CDR)

- New drug
- Common drug review
- CEDAC recommendation
  - Listing decision
  - Listing decision
  - Listing decision
  - Listing decision

Responsibility

CADTH

Drug plans
CDR process

Manufacturer responsibility

Clinical reviewers & experts

Reviews by CDR

Reviews to CEDAC for recommendation

Recommendation to drug plans

Each drug plan makes its decision

Drug plan responsibility

Submission to CDR by manufacturer

Economic reviewers

CADTH responsibility

Responsibility for CDR

Drug plan responsibility

Clinical reviewers & experts

Reviews by CDR

Reviews to CEDAC

Recommendation to drug plans

Drug plan responsibility

Manufacturer responsibility

Submission to CDR by manufacturer

Economic reviewers

CADTH responsibility

Drug plan responsibility
CDR reviews

- **Review team**
  - includes internal and external clinical reviewers, health economics, clinical experts, librarian, review manager

- **Clinical review**
  - systematic review of published and unpublished trials
  - also includes:
    - supplemental issues, background on condition

- **Pharmacoeconomic review**
  - critique of manufacturer's economic evaluation
CDR timelines

- Total process takes 20–26 weeks from submission to CEDAC recommendation
  - 1 week to review submission
  - 9 weeks to prepare reviews
  - 3 weeks for manufacturer's comments and CDR response
  - 3–8 weeks to schedule for CEDAC
  - 1 week to prepare recommendation
  - 2 weeks when recommendation is embargoed
  - 1 week to issue final recommendation
Agenda

- Introduction to Health Technology Assessment (HTA)
- HTA in the U.S. Health Care System
- HTA in Canadian Health Care System
- Principles of HTA
- Study of HTA in Emerging Markets
Essential Elements of a Technology and Outcomes Assessment Initiative (Emanuel, Fuchs, Garber, JAMA, 2007)

1. administrative independence
2. dedicated funding
3. production of objective and timely research
4. use of reliable methods
5. widespread dissemination
6. a governance and organizational structure that lend it legitimacy.
Key principles for the improved conduct of health technology assessments for resource allocation decisions

Michael F. Drummond  
University of York

J. Sanford Schwartz  
University of Pennsylvania

Bengt Jönsson  
Stockholm School of Economics

Bryan R. Luce  
United BioSource Corporation

Peter J. Neumann  
Tufts University

Uwe Siebert  
University of Health Sciences

Sean D. Sullivan  
University of Washington
One Schema for Thinking About EBM, CER, and HTA

Drummond et al.

Figure 1. Relationship between EBM, CER, HTA, and related concepts. EBM, evidence-based medicine; CER, comparative effectiveness research; HTA, health technology assessment.
15 Principles of HTA — Drummond et al. 2008

1: The Goal and Scope of the HTA Should Be Explicit and Relevant to Its Use
2: HTA Should Be an Unbiased and Transparent Exercise
3: HTA Should Include All Relevant Technologies
4: A Clear System for Setting Priorities for HTA Should Exist
5: HTA Should Incorporate Appropriate Methods for Assessing Costs and Benefits
6: HTAs Should Consider a Wide Range of Evidence and Outcomes
7: A Full Societal Perspective Should Be Considered When Undertaking HTAs
8: HTAs Should Explicitly Characterize Uncertainty Surrounding Estimates
15 Principles of HTA — Drummond et al. 2008

9: HTAs Should Consider and Address Issues of Generalizability and Transferability
10: Those Conducting HTAs Should Actively Engage All Key Stakeholder Groups
11: Those Undertaking HTAs Should Actively Seek All Available Data
12: The Implementation of HTA Findings Needs to Be Monitored
13: HTA Should Be Timely
14: HTA Findings Need to Be Communicated Appropriately to Different Decision Makers
15: The Link Between HTA Findings and Decision-Making Processes Needs to Be Transparent and Clearly Defined
Key Points from Industry Principles: Combined from PhRMA, EFPIA, and IFPMA Principles for Good Practice in HTA

• HTA should not just be applied to medicines but to all health technologies and interventions. It should be undertaken as part of a broad agenda to improve health care quality and efficiency, rather than used as a cost containment tool. Likewise “silo budgeting,” where medicines are put into a separate cost bucket, runs counter to optimising health gains across the system;

• A broad perspective of value should be used including the impact on productivity, and on caregivers and personal time, and societal health priorities, for example in terms of disease burden, should be recognised.

• HTA when applied to determine access to or reimbursement for pharmaceuticals should be kept separate from marketing authorisation.

• HTA should be inclusive, open, transparent and balanced, involving external experts and all stakeholders. It should include rights of appeal. The evaluating body should be independent of the payer.

• Payers should commit to rewarding value. Positive HTA appraisals should attract the budgetary resources necessary to fund use.

• Appraisals should recognise that value emerges through use and additional evidence over the product life cycle and recognise the need to include new data. To this same point, uncertainty around cost-effectiveness has to be dealt with in a flexible way, including the use of in-market data collection, which requires putting in place the necessary infrastructure. A full range of types of evidence including observational data can play an important role. It is important that patients get speedy access to new technologies.

• Patient preferences and needs matter in any choice of medicine. HTA guidance should give clinicians enough freedom to address individual clinical situations. In this context, the incremental nature of innovation should be recognised as should the importance of having multiple treatment options.
Agenda

• Introduction to Health Technology Assessment (HTA)

• HTA in the U.S. Health Care System

• HTA in Canadian Health Care System

• Principles of HTA

• Study of HTA in Emerging Markets
The Evolution of HTA in Emerging Market Health Care Systems

A Study by the Office of Health Economics, London and VeriTech/University of Washington, Seattle

January, 2011
Acknowledgements

• This project has been led by the Office of Health Economics (OHE) with the assistance of Professor Lou Garrison and his colleagues at Veritech Consulting and the University of Washington, Seattle
• Local researchers Michael Qin, Vanessa Teich, and Ivy Tsai have undertaken research and interviews in China, Brazil, and Taiwan respectively
• OHE / VeriTech would like to thank Stephanie Lane of PhRMA who has been the main industry point of contact for this work and her industry colleagues on the Project Steering Group
The Objectives of the Study

• To develop a categorisation of health care systems (HCS) which can be accepted by key institutions and experts in the field (WHO, World Bank and academia);

• To develop a categorisation of types of HTA using definitions recognised by practitioners in the field. Inevitably these are based on the experiences of high income countries, but can be expressed in a form that can fit into policy development in relation to the current and future healthcare systems of low and middle income countries.

• To combine these two strands (HCS and HTA) to examine the role for HTA in a health care system dependent on development stage and structure of that health care system;

• To set out these findings in a way that is helpful to understanding the potential role of HTA processes in three markets – Brazil, China and Taiwan
What exactly is HTA?

- EUnetHTA (2008) report:
  - a health technology is ‘any [health] intervention that may be used to promote health, prevent, diagnose or treat disease, or for rehabilitation or long-term care. This includes pharmaceuticals, devices, procedures and organizational systems used in health care’

- We can categorise HTA into three types:
  - “micro-level” HTA aimed at appraisal of individual technologies, or groups of related technologies
  - “micro-level” HTA aimed at developing clinical practice guidelines or the way in which individual technologies are combined within a delivery system to manage patients efficiently
  - “macro-level” HTA which is about the efficiency of the organizational systems or architecture of the health care system
The conceptual model

- **LEVEL OF SPEND**
  - What quantity of resources are available?

- **DEGREE OF CENTRALISATION**
  - Who makes decisions about what health care is funded?

- **FOCUS OF HTA**
  - What is appraisal concerned with?

- **BREADTH OF HTA**
  - Which health services are appraised?

**OUTCOME OF HTA**

- Existing bundles of services and service delivery
- Existing regulatory and reimbursement mechanisms
Health care system typology: two key attributes/variables and levels

**LEVEL OF SPEND**

What **quantity of resources are available?**

- Low spend per capita
- Medium spend per capita
- High spend per capita

**DEGREE OF CENTRALISATION**

Who makes decisions about what health care is funded?

- Out of pocket spend dominates
- Emergence of insurance /collective funding; decisions localised
- Active third party purchasing

PhRMA 2011: AMACS Meeting
Degree of Centralisation

- The extent to which there is third party coverage and so an interest in the use of both “micro” and “macro” technologies that goes beyond the provider-patient relationship that dominates an out-of-pocket spend environment.
- The extent to which there is active rather than passive purchasing by the third party insurer. Related to this is the degree of national level regulation as to what is included in the insurance package offered to enrollees.
HTA typology: key HTA system attributes/variables and levels

**FOCUS OF HTA**
What is appraisal concerned with?
- Efficacy/safety
- Relative effectiveness
- Cost-effectiveness (C-E)
- C-E and broader issues

**BREADTH OF HTA**
Which health services appraised?
- Basic preventative services and minimum care packages
- New technologies
- All technologies/services

PhRMA 2011: AMACS Meeting
Observations (i)

- Observation 1: Incomes are growing in emerging markets, but resulting increases in funding for health care are likely to be out-paced by rising demands and expectations. In such situations, HTA may have a role in assisting the health care system to reconcile rapidly expanding demand with more slowly expanding resources. HTA can provide a potential means of handling this in a more explicit and transparent way, and in promoting public debate about priorities.
Observations (ii)

• Observation 2: HTA of individual technologies is not a substitute for the reform of health care systems. Where health care systems create obviously bad incentives, this type of micro-HTA is unlikely to compensate for these failings.
  • HTA should not be approached out of context. HTA should be tied, in a case-by-case way, to what else is going on in the health care system.
• Observation 3: ‘One size fits all’ HTA processes and methods are unlikely to be appropriate for emerging markets. There needs to be clarity over the purpose of HTA – and the methods and processes which are adopted need to be fit for purpose.
  • HTA is not an objective ‘tool kit’ that is transferable to any setting.
  • “Value” of new drugs varies, and is subjective and based on local preferences and other values.
  • Real value depends in a “second-best” world on the match between costs and the value of all other inputs (hospitals, physician, nurses, equipment, etc.).
Observations (iii)

• Observation 4: HTA and pricing regulations work hand in hand: the approach to HTA should be appropriate to, and work sensibly in combination with, the particular approach to pricing technologies.
  • For example, HTA based on reimbursement levels ignores what providers actually have to pay for new drugs. This would tend to under-estimate real-world cost-effectiveness
• Observation 5: There is no single prescription for HTA methods and processes which will be welfare-increasing in all contexts.
  • Further, trade-offs between competing objectives are likely if not inevitable; and health care systems may differ in the relative value placed on them, for example, the achievement of equity goals; technical efficiency; cost containment; and patient choice. Every health care system is on a slightly different trajectory: as it develops, and as spending increases, the way that HTA evolves will be a reaction to the possibilities and pressures that new technologies present.
  • The key message is that the relevance and positioning of any role for HTA in a health care system depends on the development stage and structure of that health care system.
HTA and International Reference Pricing (IRP)

• Compliance with good (micro) HTA principles is resource-intensive for governments (and industry.) Countries may “free ride” and some evidence does cross boundaries. However, other evidence does not.

• Alternative “low resource” options such as IRP may be less efficient when reference countries have different income levels and willingness to pay for health gain.

• Therapeutic RP is also “low resource” as it assumes treatments are the same, rather than looking at evidence.

• Efficient local (micro) HTA may be more efficient than IRP and TRP if it respects good HTA principles, and reflects local willingness to pay for health gain.
Final Observation

• The impact of HTA and cost-effectiveness information on health system resource allocation depends on:
  
  – **Incentives** to use the information
  
  – Insulation from political influence
    
      (−or at least a fair and workable system of checks and balances.)

Final question for discussion:
What would be “efficient” HTA in Taiwan? From a Taiwanese perspective--Short-term (static) vs. long-term (dynamic)?
Thank you!

Questions?

Lgarrisn@uw.edu