

# ***From Evidence-based Medicines to Value-based Medicines: What's in this Evolution?***

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# Outline

- The history of transition
- What is “value” when it comes to medicines?
- How do we measure “value”?
- Recent trends of development of value-based assessment in some Asian countries
- Is “value-based” approach the final answer? What are the challenges
- Looking ahead and Conclusion

# **The history of transition**

# Background

## Economics of healthcare

- “Supply and demand”: if the price of a commodity is too high, consumer demand will decrease and supply will therefore consequently decrease too and vice versa
  - “Elasticity”
- Healthcare spending by individuals is “inelastic”: does not follow the normal “supply and demand” rule e.g. no matter how expensive insulin syringes are, diabetic patients would need to purchase it or find way to do so
- Demand is always on the rise, yet prices are also increasing, sometimes even faster than inflation rate
- Somehow some approaches will need to be in place to ensure demand is met (accessibility), patient still afford to purchase (affordability), and the system can continue (sustainability)

# Evidence-based medicine (EBM)

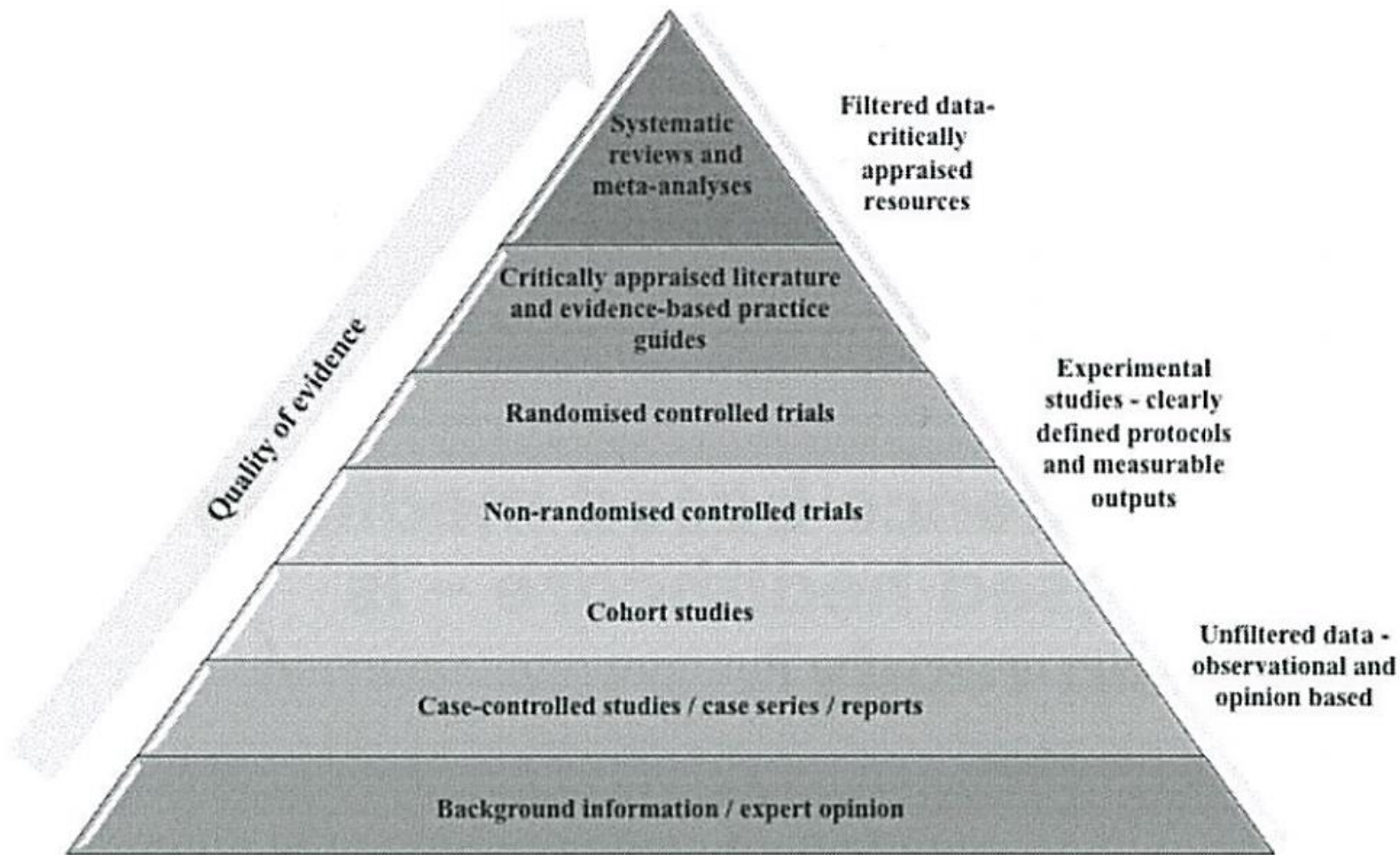
- Core of clinical decision making starting in late 1990s
- Decision is based on best available research information rather than solely on a clinician's expertise, so as to minimize the uncertainties of clinical examinations (Bae 2013, 2014)
- Quality of evidence : 5-level system

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Level 1	Randomized clinical trial with Type I error $\leq 0.05$ and Type II error $\leq 0.20^*$
Level 2	Randomized clinical trial with Type I error $\geq 0.05$ and/or Type II error $\geq 0.20^*$
Level 3	Non-randomized clinical trial
Level 4	Case series
Level 5	Case study

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TABLE I. Levels of interventional evidence.<sup>24</sup>



([http://s3-ap-southeast-2.amazonaws.com/crcaustralia/wp-content/uploads/2017/04/11055225/figure1\\_bis.jpg](http://s3-ap-southeast-2.amazonaws.com/crcaustralia/wp-content/uploads/2017/04/11055225/figure1_bis.jpg))

Adapted from an evidence based medicine pyramid generated by Dartmouth College (6) and Central Michigan University (7).

# Evidence-based Medicine

## Concerns:

- Efficacy from clinical trials vs clinical effectiveness
- Patient offered a hurried explanation about treatment options, obtain written consent, decision made
- Patient's preference not taken into decision-making process
- Despite a GDP per capita healthcare spending of USD5,000 (twice as much as other industrialized countries), the US only ranked 37 out of 191 in overall effectiveness and 72 in efficiency by WHO (WHO 2000)
- As healthcare cost increases, questions arise:
  - are we paying the right cost for the service
  - are we able to maximize the benefits with our spending
  - is the level of spending sustainable



# Value-based medicine (VBM)

- “VBM” was introduced in early 2000s
- Concept: Using the best evidence-based data and converting it to patient value-based data to deliver patient care that goes beyond EBM
- Definition: The practice of medicine incorporating the highest level of evidence-based data with the patient- perceived value conferred by healthcare interventions for the resources expended (Brown 2005)
- 3 components of VBM
  - use of best research evidence available
  - patients’ values are converted into measurable parameter called “utilities”
  - cost-utility level expected from a treatment option forms the basis for decision-making



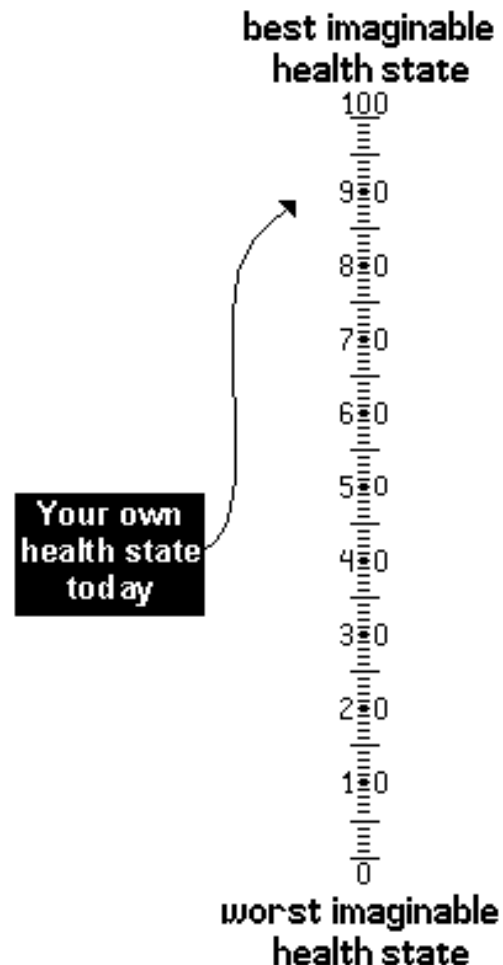
*Fig. 1.* The value-based medicine pyramid.

# VBM (Cont'd)

Operationally, VBM is carried out in 3 steps

- Quantifying improvement from an intervention
- Data converted to “value” form using utility analysis
  - by instruments such as SF-36 or EQ-5D/EQ-5D 5L (function-based)
  - by patient’s preference e.g. time tradeoff, standard gamble, willingness-to-pay (preference-based)
- Incorporation of cost of intervention with the value conferred using cost-utility analysis

# EuroQol EQ-5D: Utility value



- **MOBILITY**
  - I have no problems in walking about
  - I have some problems in walking about
  - I am confined to bed
- **SELF-CARE**
  - I have no problems with self-care
  - I have some problems washing or dressing myself
  - I am unable to wash or dress myself
- **USUAL ACTIVITIES (e.g. work, study, housework family or leisure activities)**
  - I have no problems with performing my usual activities
  - I have some problems with performing my usual activities
  - I am unable to perform my usual activities
- **PAIN/DISCOMFORT**
  - I have no pain or discomfort
  - I have moderate pain or discomfort
  - I have extreme pain or discomfort
- **ANXIETY/DEPRESSION**
  - I am not anxious or depressed
  - I am moderately anxious or depressed
  - I am extremely anxious or depressed

**What is “Value” and how it is measured**

# Price vs Value

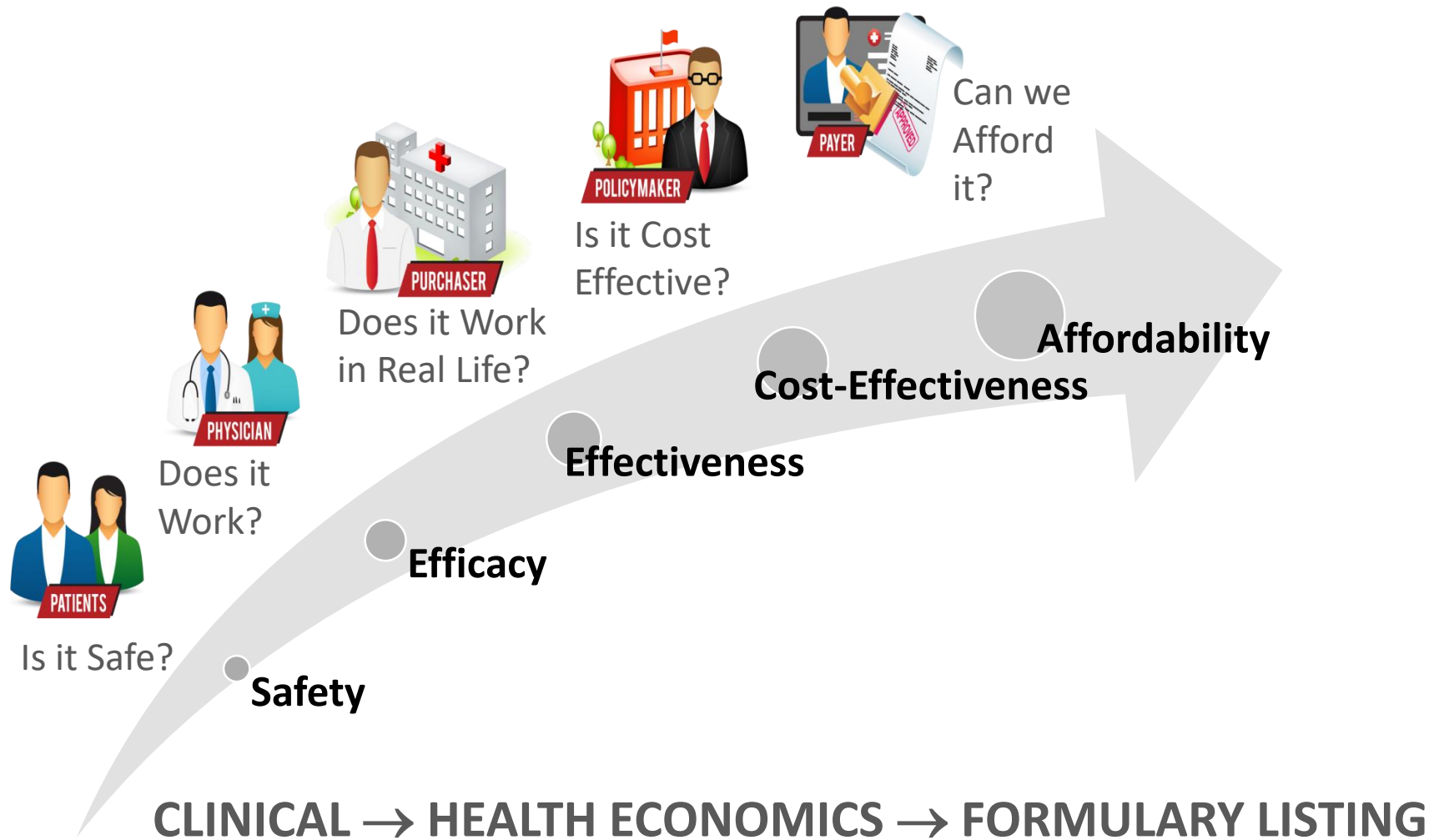
## Price

- Fixed
- Easy to count
- Visible
- e.g. unit price of a drug/therapy

## Value

- Difficult to estimate
- Difficult to demonstrate
- Humanistic consideration included
- Examples: quality-of-life, overall effect on the society, impact on the treatment strategy, impact on epidemiology etc

# Steps to establish “value” of a medicine

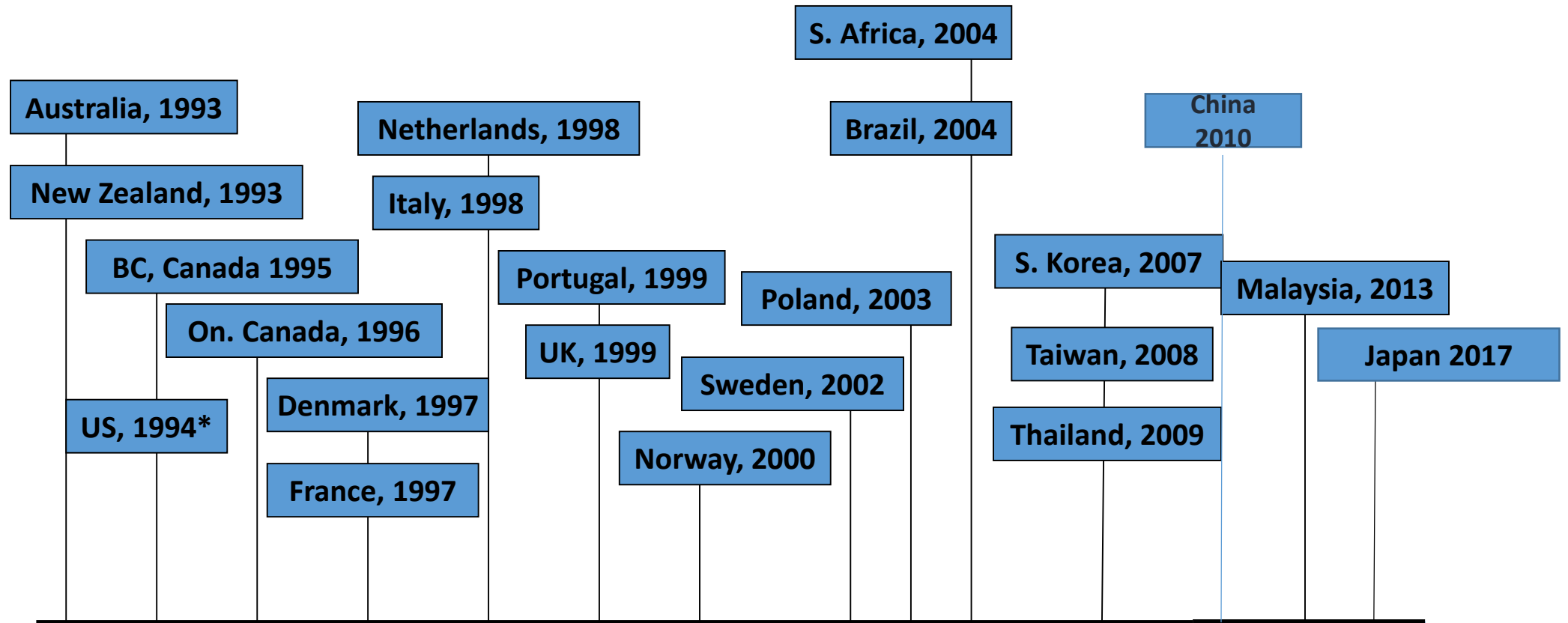


# Applications of value-based assessment

- Assessment of new health technologies in the form of Health Technology Assessment (HTA) leading to registration, formulary listing, price-setting etc
- Development of disease management guidelines



# Formal use of value-based assessment for new pharmaceuticals around the world



# How is “value” measured?

- Concept of Quality-adjusted life-year (QALY) introduced in 1968 by Klarman et al for renal disease managements
- Reason: difficult to compare costs and benefits of treating different conditions by traditional bio-indicators e.g. comparison of benefits in treating cancer vs mental illnesses
- QALY: able to compare effectiveness between different conditions
- Cost effectiveness ratio:  $\text{Cost}/\text{QALY}$  gained
- Incremental cost effectiveness ratio (ICER):  $\text{Increase in cost}/\text{Increase in QALY}$

# **Recent trends of development of value-based assessment in some Asian countries**

## S. Korea



- Introduced in April 2007 as a component of health care reform
- Aims to assess the safety and effectiveness of new health technology
- To encourage the development of high technology in health care sector
- Organizations involved: National Health Insurance Corporation (NHIC) and Health Insurance Review Agency (HIRA)

# Taiwan



- Division of HTA was established in late 2007 under the Centre for Drug Evaluation (CDE), a non-profit-making NGO funded by the DoH of Taiwan
- The division became operational in early 2008
- Major function: to provide evidence report to Bureau of National Health Insurance (BNHI) for every new drug submitted for reimbursement
- Cost-effectiveness data is not specifically required in submission, but local study/data will be rewarded

# Thailand

- Starting from 2007, with the establishment of the HE Working Group, listing as an essential drug/health technology requires cost-effectiveness evidence
- 2007 Health Intervention and Technology Assessment Program (HITAP): independent research institute funded by MoPH
- 2 committees under HITAP: list of essential medicines and HE working group



# Singapore



- MOH began conducting HTAs to inform policy-making in 1995 under the Pharmacoeconomic & Drug Utilisation Unit (PEDU)
- Agency for Care Effectiveness (ACE) established in April 2016
- One of the main missions: To advance the values of evidence-based practice and appropriate care
- Value-based pricing incorporated in evaluation process

# The Philippines

- Philippine Health Insurance Corporation (PhilHealth) established the HTA Committee in 1999 for development of reimbursement policies
- HTA Committee's 3 major functions:
  - conduct assessments to determine drugs for reimbursement
  - development of clinical practice guidelines
  - evaluation of effectiveness and safety of procedures





# Indonesia

- 2012 HTA committee established by MoH to evaluate procedures, drugs and devices to achieve cost effective use of resources and improve quality of care
- Guideline in 2012
- Pricing managed by tendering, negotiation and risk-sharing schemes for innovative medicines
- Since 2014, HTA Committee has been conducting appraisals on drugs and devices





# Malaysia

- Malaysia Health Technology Assessment Section (MaHTAS) established in 1995 under MoH to provide input for policy developments
- Formal requirement for HE data (mainly budget impact) for new drugs implemented in 2016 by MoH
- Ongoing discussions on innovative market access schemes such as risk sharing, performance-based, managed entry agreements (MEA) in recent years

**Is “value-based” approach the final answer?  
What are the challenges?**

# Pros and Cons of VBM

## Pros

- Patient's value and quality of life embraced
- Congruent with medical ethics and capable to reducing uncertainties of clinical decisions
- Improves efficiency of resource allocation by prioritizing options
- Capable to maximize benefits from spending
- Sometimes can lead to savings e.g. 7% (USD 115B) savings in health care expenditure recorded in the US while improving QoL at the same time (Brown 2000)

# Pros and Cons of VBM (cont'd)

## Concerns

- Definition of “value” varies
- Whose “value” are we measuring? Quantitatively, “value” varies among different stakeholders e.g. patient populations, service provider, payer, insurance provider, hence potential conflicts among stakeholders
- “value” changes over time
- Comparisons between countries difficult due to difference in health care system and economic structures
- Difficult to measure value for interventions that are preventive in nature or long term in duration

# Pros and Cons of VBM (cont'd)

## Concerns

- Threshold of cost-effectiveness is not equivocal e.g. a University of York study revealed that the UK threshold could be as low as £13,000/QALY (vs £20,000/QALY) (Claxton 2015)
- Availability of options with high value not always guaranteed
- Universal database of utility values not available
- Healthcare budget does have a limit

# Ad hoc approach in defining “value”

- Gavi (vaccines financier of the developing world): Cost per death and case averted (potential double counting and preference based)
- Global Fund to Fight AIDS, Tuberculosis and Malaria: Spend reduction in commodity purchases made within the pooled procurement mechanism for equivalent commodities at equivalent quality and volume
- DFID of UK: Impact on poverty reduction relative to the inputs... invested in it (alternative use not considered)

# UK NICE experience

- Originally set up in 1999 to standardize availability and quality of NHS services
- 2005 name changed to National Institute for Health and Clinical Excellence (2013 to National Institute for Health and Care Excellence) to develop guidance and quality in social care, accountable to the Department of Health and Social Care
- Value has always been measured in terms of QALY
- 2013 NICE was asked to introduce “other aspects of value” into their decision making e.g. burden of illness, and other wider societal effects such as loss in productivity



## UK NICE experience (Cont'd)

- NICE's view: "productivity" favoured patients of working age hence elderly and children disadvantaged
- "Absolute QALY difference" between patients receiving conventional care for a condition and the equivalent population without the condition
- A measure of reduced capacity of patients to engage with society as a result of the condition
- Wide consultation but feedback received was conflicting and largely negative
- NICE's conclusion: Proposed change did not adequately reflect differential value for new therapies and varying benefits to wider society, "status quo" recommended

## Hence may affect

- Value-based access schemes
- Evaluation methods such as multi-criteria decision analysis (MCDA)
- Value-based pricing methods
- ICER of new health technologies
- Generalizability of study results

# Lessons learned from some western countries

- Developed with good intentions but underlying principles of economics often ignored
- Inadequate testing and conceptual piloting in development of tools for value measurement
- Public support is important for policy implementation
- A wider societal consensus on “value” is necessary
- Capacity building is imminent

## Lessons learned from some western countries (Cont'd)

Key success factors for VBM (BCG 2012)

- Clinician engagement: as lead in collecting and interpreting data
- National infrastructure e.g. shared IT platforms, patient data protection
- High quality data via disease registries
- Outcome-based incentives to realign incentives to optimize care: so that care quality can be improved

# Summary and conclusion

- Value-based approach will still be the global standard for assessment of new health technologies
- More and more new concepts and ideas will emerge
- Before embarking on VBM, one must ensure all the infra-structures e.g. data resources, registries are in place
- “Think it through”
- Beware of fake values e.g. false savings from cost shifting and sub-standard restricted services
- Capacity building is imminent
- Wider society must be involved in discussion