

IP2 Opportunities and Challenges in International Harmonization of HTA of Medical Devices – Gaps Between European and Asian Countries.. From Japanese view

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HTA (narrower definition) in Medical devices in Japan

- Approved by PMDA, reimbursed via MHLW, Chu-i-Kyo
- Dossiers for reimbursement contains;
 - Possible numbers of patients and sales amount
 - documents for “Health economic usefulness” (**Legacy HTA**)
- Several products (not new but current existing) are nominated as candidates for the **Newly-developed HTA program** since Apr. 2016

State-of-the-art HTA vs Legacy HTA

- A few devices are nominated for pure HTA submission as the “Pilot HTA introduction” in Japan
- For a long time, the dossier already contains “usefulness from the Health-Economic perspective”

Requirement for “usefulness from HE”	
A	Additional medical cost with introduction of new devices
B	Medical cost saved with introduction of new devices
C	Overall impact for medical costs (A minus B)

Device vs Drug??

- Device HTAs is more likely to be bothered with..

Lack of data	No (Scarce) RCT is available, while there are some observational studies
”Value” of data	”New” device, which was nominated for HTA submission, will no longer be “New” one as next-generation ones will soon become available
Lack of capacity	Some device is suitable for CUA, while others not

Uniform guidelines both applicable for drugs and devices

- **Issues around data sources**
 - Data prioritization (Scarce RCT vs. Sufficient Obs. Study)
 - Capability of CUA (QALY preferred)
- **Scarce availability for DIRECT comparison**
 - Devices **MOSTLY REPLACED** one should be the comparator
 - Rapid replacement (comp. drugs)

Assessment results of MSAC australia (devices for treatment)

- 101 Results are available for 1999-2017

	Methods	Number
Economic Evaluation Performed 55 (54%)	CUA (with QALY)	22
	CEA (without QALY)	14
	CMA	12
	CUA and CEA	6
	CUA and CMA	1
Not performed 46 (46%)	cost comparison/cost analysis	19
	nothing	26
	PBAC did	1

Characteristics of JP-HTA (pilot)

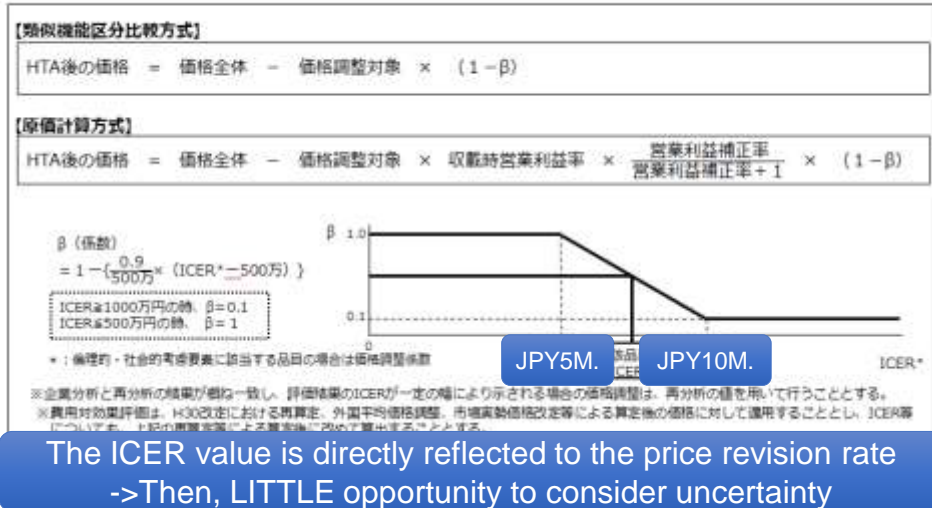
1	Eligible products are chosen from drugs ALREADY REIMBURSED
2	Results are used for PRICE REVISION , not for COVERAGE DECISION (French HAS – like system)
3	HTA result will be applied only to PREMIUM portion
4	ICER values are compared with the threshold value to determine if it is cost-effective (UK NICE – like system)
5	The threshold value will be defined via several survey , including WTP (What is often referred to in basic textbook)
6	Things other than Cost-Effectiveness will be taken into account at the appraisal process (UK NICE – like system)
7	Drugs with multiple indications are evaluated via merging multiple ICER value (ORIGINAL system)

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Japan-specific way how to reflect results into price revision rate

(図4) 価格調整方法



9

How can we justify JPY5M. and JPY10M?

• The function of Multiple threshold values

	How multiple threshold values are used?
Foreign country (UK, Netherlands)	Threshold value is chosen among multiple ones, according to the characteristic of diseases/drugs Values would be varied one intervention to another
Japan	Two "Threshold values", JPY5M and 10M will be applied to ALL candidates

How the “other factors” could be taken into account at Appraisal phase?

Component	Description
Public health matter	”External usefulness” like herd effects for communicable diseases
Costs other than HC payers’ perspective	Caregiving costs and productivity losses should be taken into account in some particular cases
Disease severity	”End-of-Life” like issues?
Availability of alternative treatment	In order not to prevent the development of treatment for diseases which no alternatives are available
Innovation	To enhance the development of innovative/novel medications
Pediatric disease	To hold the marketability of pediatric medications

ICERs will be discounted for 5% per 1 criteria met..
(cf. End-of-Life in UK: 20K-30K to 50K)

No additional factor needs to be considered in the appraisal process???

- What is the key role of the appraisal?

Viewpoint	Role	Importance
Practical	Simply minimize price reduction rate	Less important Additional factor should only be considered if HTA is used to coverage decision
Conceptual	To compensate the limitation of CEA/ICER	More important Other factors should be seriously considered, as no flexibility is allowed for CEA/ICER part

“Extra value” other than CEA/ICER is difficult to be incorporated to one-dimensional scale

Issues around traditional “evidence level”

- Which one should be preferred?
 - Scarce direct comparison data (RCT)
 - Indirect comparison data
 - Meta-analysis of observational data

NO clear-cut criteria for prioritization

Several challenges around Japanese HTA

Challenges and Best Practices for the Japan Health Technology Assessment Pilot Program

Five Bjarth Kristensen, MD, PhD, University of Southern Denmark, Odense, Denmark; Akira Igarashi, PhD, University of Tsukuba, Japan; Peter J. Neumann, ScD, Tufts Medical Center, Boston, MA, USA; Dawn P. Goldman, PhD, University of Southern California, Los Angeles, CA, USA

KEY POINTS

Health technology assessment (HTA) processes continue to expand globally.

Advancing identifying new HTA processes that fit governments from the perspectives of existing HTA bodies, adapted to regional circumstances.

These guiding principles may be helpful for Japan, which is in the process of developing and establishing their own HTA.

To guide health technology policy decisions, countries often rely on health technology assessment (HTA). HTAs typically involve the evaluation of a medical technology's impacts, but can be specialized for different purposes, depending on the needs and policies of a country. For example, countries such as the United Kingdom, Canada, and Australia have used HTAs to inform resource allocation in the new context of healthcare cost concerns, a lack of countries without formal HTAs, such as Japan, China, USA, and Argentina, are implementing their own concepts.

process that evaluates drugs and medical devices cost-effectiveness (clinical and economic benefits) relative to a comparable technology. In 2018, the HTA pilot phase evaluated 7 previously marketed drugs and 6 medical devices. The results of the HTA evaluation will be reflected directly by a price revision, but only a portion of the price can be adjusted via the HTA review. For other factors, including social impact and ethics, may be considered during the appraisal process, each assigned a 0% weight. Although the results of this pilot have

When not executed according to sound principles, HTA could be viewed skeptically as an attempt to limit patient access to certain costs or a lever for government price negotiation.

For countries developing their own HTA systems, the variability in HTA purpose and structure can raise identifying optimal aims and processes challenging. For example, high- and upper-middle income countries tend to use HTA to guide reimbursement and coverage decisions, lower income countries tend to use HTA for planning and budgeting [1]. In this piece, we discuss some common challenges and best practices for designing HTAs to address, particularly

that have been discussed publicly. Japan aims to formally launch their HTA in April 2019.

COMMON CHALLENGES FOR NEW SYSTEMS AND PROCESSES OF HTA

The first step in establishing an HTA is to determine the policy objectives that the HTA should address. These decisions depend on many people health and economic factors, such as healthcare coverage. Since Japan's health system covers all residents,

DEFINING A COST-EFFECTIVENESS THRESHOLD

HTAs commonly rely on cost-effectiveness thresholds, or the cost for each additional unit of the outcome where “cost-effectiveness” or reasonable cases is anticipated. This benchmark can also be increased or decreased to reflect the opportunity cost of the health outcomes for the marginal intervention that must be relinquished to provide resources for a new intervention [4]. Typically, a quality-adjusted life year (QALY)—a measure of the duration and quality of life—can be used in the cost of lifetime.

Since cost-effectiveness thresholds can inform whether a technology has “low” or “high” value, understanding the appropriate value is essential. There are no universally accepted values, but some fall in the range of one to three times gross domestic product (GDP) per capita. In the United States, for example, the conventional willingness-to-pay range has been \$50,000 to \$150,000 USD, and in the United Kingdom, it has been closer to \$25,000 per QALY [2]. In both countries, thresholds may be higher for low-value or targeting certain diseases or populations. Japan's currently selected threshold of JPY5M falls within the lower end of the range. However, the function of the threshold could be considered conceptually different as a starting point for price negotiations, instead of as a benchmark for coverage decisions.

BUILDING CAPACITY

In addition to the scientific methodology, developing a digital HTA site requires identifying and allocating human and financial resources to support the process. Securing and retaining these resources can be challenging, especially for countries without established HTA programs [1]. Engaging global experts during the HTA development process can help ensure that lessons regarding an assessment and knowledge.

STAKEHOLDER BUY-IN

When not executed according to sound principles, HTA could be viewed skeptically as an attempt to limit patient access or a lever for government price negotiation. Involving a range of stakeholders, such as patients, providers, manufacturers, and academic experts in the development process can support the credibility of an HTA.

TRANSPARENT PROCESSES AND DECISION MAKING

Many HTA guidelines recommend transparent processes and decision making for the evaluation of new medical technologies. [1,3]. Transparency can increase appropriateness of the approaches selected, build greater confidence in the results, and allow for greater participation of all stakeholders, including manufacturers, providers, and patients.

REAL-WORLD DATA INCORPORATION

Randomized controlled trials remain the gold standard for evidence of efficacy and safety for new medical technologies. However, there is growing interest in incorporating real-world data, given differences in patient populations and behaviors between the controlled setting of clinical trials and the real world.

MULTI-STAKEHOLDER ENGAGEMENT

There has also been a growing recognition of the limitations of conventional economic methods to measure the true value of new medical technologies. For example, a treatment's nonclinical benefits, such as reduced caregiver burden and improved productivity, are often not included in standard economic evaluations. Incorporating real-world observations, particularly patients, are included can help ensure that the full benefits of a treatment are assessed.

Through the implementation of its HTA pilot, Japan's MHLW has taken an important first step in developing a scientifically based system that can inform the optimal use of its limited resources. As Japan and other countries establish new HTA processes, support from global experts in HTA will be valuable to ensure that lessons learned from other countries can be leveraged and operationalized.

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REFERENCES

1. Health Equity International. Global survey on health technology assessment by

Kristensen KB, Igarashi A, Neumann PJ, Goldman DP. Challenges and Best Practices for the Japan Health Technology Assessment Pilot Program. ISPOR Value & Outcomes Spotlight 2018; 4 (4): 40-1.

My personal view for HTA

It should be “SUICIDE OF ACADEMICIAN”, if I say

Japanese HTA is fairly good system, as EXPERTS says that “it is fairly good system”.

Should there be any issues, we have only to say “further discussion will be very important”, regardless of the actual capability of discussion

PRE-HTA ERA is more favorable. Then, we need to go back to that ERA. We can deceive the publics, only arguing that “ACCESS LIMITATION”!