European Federation of Pharmaceutical Industries and Associations

EFPIA Japan's Basic Position on Cost-Effectiveness Assessment in Japan

"Delivering Patient-Centered Innovation"

EFPIA Japan

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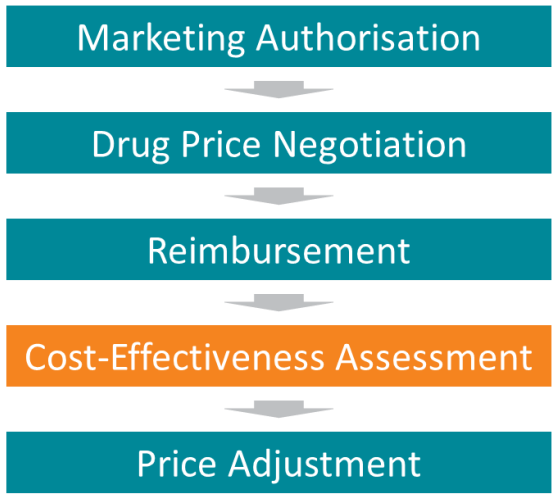
EFPIA Calls For Continuous Review of Cost-Effectiveness Assessment

In April 2019, the Government of Japan officially introduced "cost-effectives assessment" (CEA) for pharmaceuticals and medical devices after its pilot program was completed. As Japan's version of health technology assessment (HTA), the newly introduced system is aimed to adjust reimbursement prices of pharmaceuticals (drug prices) after product launch.

Until recently, drug prices in Japan were decided only according to evaluations of innovativeness and other factors, based on efficacy and safety data. However, since April 2019, the new system also reviews drug prices from an economic viewpoint that examines "cost" and "effect" relative to the comparator, when the government's criteria are met or the government recognises the need for the review. (See Figure 1)

Japan has limited experience in HTA, and therefore needs to enrich its expertise in health economics. Since the official introduction of the new system, European pharmaceutical companies and officials of the government's HTA body have held a series of discussions on selected medicines for CEA. However, there have been different views on academic expertise and due processes between the pharmaceutical industry and the government HTA body. We believe more transparency in the CEA process and better systems are needed for objective discussions.

Figure 1. CEA process for new medicines (if selected)



Ethical concerns in rare disease treatment

The Ministry of Health (MHLW) has set a basic rule that excludes from CEA treatments only for rare diseases with small patient populations or with paediatric indications. However, it also allows that "the Chuikyo may make decisions on what should be selected for CEA" if the expected sales forecast is large and the unit price of the drug is significantly high[1]. The Chuikyo (Central Social Insurance Medical Council) is an advisory board for the health minister, whose officials are drawn from healthcare professionals groups and payer association representatives.

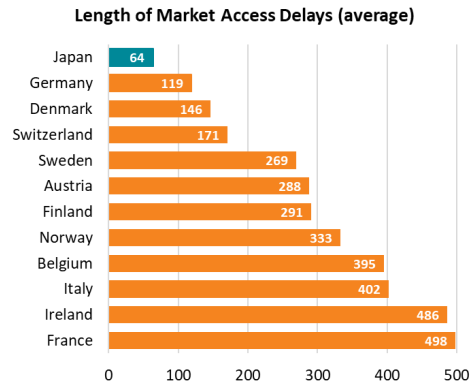
The MHLW has selected several new medicines for CEA since April 2019, which includes "orphan drugs" for leukaemia and other rare disease.[2] This means "cost-effectiveness" in treatments for diseases with small patient populations and high medical needs is requested to be proven. We believe rare diseases need to be completely excluded from CEA for ethical reasons, because Japan's universal healthcare coverage highly values the basic principle of equality in healthcare services.

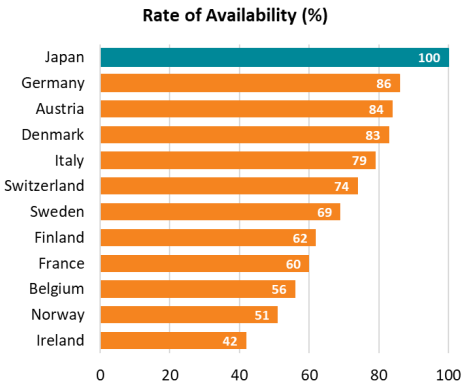
"Drug lag" concerns again; denial of drug reimbursement?

Some stakeholders in Japan recently proposed that reimbursement decisions for medicines should be made after CEA. The Ministry of Finance, for example, seeks to use CEA for reimbursement decisions in the future.[3] A representative of a payer association also shows a similar position.[4]

The MHLW, however, still maintains its traditional position that in principle all the pharma-

Figure 2. Patient Access to Medicines in Europe and Japan





Length of Market Access Delays: 2018 study: 2015-2017. The average time between marketing authorisation and patient access, measured by the number of dayselapsing from the date of EU marketing authorisation (or effective marketing authorisation in non-EEA countries) to the day of completion of post-marketing authorisation administrative processes. Waiting times reflected in the Patients W.A.I.T. Indicator include any delay, whether attributable to companies or to competent authorities. Fundamentally the "waiting time" in Japan is 60 days and as only the timing of coverage by reimbursement for price revision is 90 days, the weighted average of these values are used in this analysis.

Rate of Availability: measured by the number of medicines available to patients in European countries as of 2018: for most countries this is the point at which the product gains access to the reimbursement list.

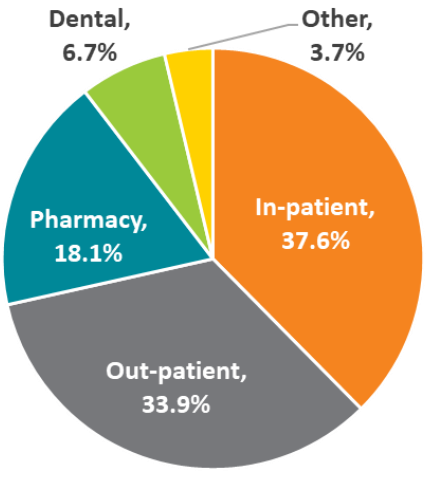
Source: EFPIA Patient W.A.I.T. Indicator 2018 Survey

ceuticals should be reimbursed in the public insurance system after marketing authorization. The ministry says it is difficult to gain public support for changing the principle.[5] A major physicians' group also shares a similar position.[6]

Japan has a principle to make decisions within 60 days on reimbursement of medicines newly authorized to market, making patient access possible in a timely manner. However, in other countries, patients cannot always access new medicines in a timely manner and fail to enjoy the best treatment due to reimbursement denial after HTA.[7]

The waiting time for patient access to new medicines after marketing approval varies country to country in Europe (See Figure 2). If Japan introduces a similar policy to make reimbursement decision based on CEA for new medicines, its world's best healthcare services where everyone can benefit from the latest and quality treatments may drastically change in a negative way. The drug lag issue, which has been solved by the greatest efforts by the government[8], may emerge again in Japan.

Figure 3. Composition of National Medical Expenditure (%)



Source: MHLW "Summary of National Medical Expenditure in FY2017"

Efficiency in healthcare, total optimization needed

Japan's national medical expenditure has now reached 43 trillion yen[9] and is expected to continue to grow. The government has been working on social security reforms to control the increasing expenditure in Japan's aging society.

Pharmaceutical spending accounts for about 20% of the total national medical expenditure, which is an unchanged trend for years in Japan.[10] The other expenditures come from in- and out-patient services as well as pharmacy costs (See Figure 3).

We believe continuous efforts to balance the value of innovative medicines and the sustainability of Japan's universal healthcare coverage through total healthcare expenditure optimisation are important. All the stakeholders in healthcare such as patients, healthcare professionals, policy makers and the pharmaceutical industry need to seek the best solution based on accurate and scientific evidence.

Social values of medicines; Future cost reduction effect

The value new medicines bring to patients is not limited to clinical outcomes. Their social values such as improvement in Quality of Life (QoL)

and reducing caregivers burden also contribute to reduce the total disease burden that benefit both patients and society.

Some cancer medicines have easier administration than conventional ones, as well as proven efficacy to extend patients' survival. As the cancer population increases while survival rates have been improving in Japan[11], new cancer treatments are to expected to allow more patients with cancer live longer and to remain active in the workforce .

Medicines for stroke prevention have benefit not only for patients' health outcomes but also for reducing the care-giver burden because the treatments can reduce the risk of being bedridden after stroke. It is encouraging that the government legislated a new law for stroke and cardiovascular disease prevention[12] because stroke, the 3rd biggest cause of death in Japan[13], and its prevention are one of the most prioritised social issues in Japan.

COPD (chronic obstructive pulmonary disease), whose primary cause is smoking, is a lifestyle disease for people in middle age[14]. It is also reported that the lost weekly working time in COPD patients is significantly longer than for healthy people[15]. Treatments for diseases with negative impact to people's socio-economic activities like COPD are expected not only to improve patient health status but also reduce social loss.

New medicines with improved efficacy, safety and utility profiles will contribute to solve social issues and offer improved QoL for patients. We believe that these significant additional values new medicines bring about need to be assessed appropriately and transparently.

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EFPIA's Principles

We, EFPIA Japan, call for continuous efforts to review the CEA scheme by the government, and propose the following principles.

1. **CEA should NOT be used for reimbursement decisions.**
2. **CEA should be used only as a complementary tool for drug pricing and reimbursement systems.**
3. **The ethical and social values of pharmaceuticals should be evaluated from a long-term perspective.**
4. **All stakeholders in the CEA processes should be involved in a transparent manner.**

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European Federation of Pharmaceutical Industries and Associations, Japan

EFPIA Japan was established in 2002, and represents 23 R&D-based European pharmaceutical companies operating in the Japanese market. We are the voice of the European innovative pharmaceutical industry in Japan. Our mission is to contribute to the welfare of the Japanese population through the provision of innovative pharmaceuticals and vaccines without delay.

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