Pharmaceutical Association of Malaysia
Persatuan Farmaseutikal Malaysia

Value-based Medicines for Improved Patient Access in Malaysia White Paper

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Executive Summary

The mid-term review of the 11th Malaysia plan aims to achieve universal access to quality health care while also ensuring sustainability to the health system. The focus must shift to providing value: increasing treatments, products and services that are both clinically effective and efficient.

In order to achieve these goals, PhAMA recommends the following factors be reviewed and considered:

- Equity, Efficiency and Effectiveness be considered in the context of health care decision making in Malaysia
- Alternative methodology or pathway be evaluated for articulating cost-effectiveness and promoting equity in health care
- A review be conducted of the threshold approach that is currently being applied in Malaysia (and the associated challenges)
- The World Health Organisation (WHO) position on cost effectiveness thresholds that place Incremental Cost-Effectiveness Ratios (ICERs) in the context of other public health options available or already adopted in the relevant Malaysia setting – and in the context of budget be reviewed
- New frameworks like Multi-criteria Decision Analysis that could offer an improvement on the use of simple thresholds and its relevance to Malaysia be considered

Through our collaboration with the Pharmaceutical Services Programme and other stakeholders, it is evident that sustaining progress in health care in a time of cost containment requires commitment, multi-levelled engagement, and strong leadership while keeping the patient in the center of access.

PhAMA is committed to engage in early dialogues with Health Technology Assessment (HTA) bodies/payers to best fit their expectations for value added medicines development and obtain recognition of additional value through flexible and innovative initiatives.

This white paper does not presume to offer a strict set of policy prescriptions; rather it is intended as a call for action of future directions to guide policy makers entrusted with improving health care and wellbeing of Malaysians in line with its goal under the mid-term review of the 11th Malaysia Plan and globally, its commitment to Sustainable Development Goals (SDG).

Background and Context

The Malaysian health care system is rightfully considered as a regional success story among countries of equivalent socio-economic status. Malaysia has achieved Universal Health Care, although established UHC needs to be continually evaluated with respect to its key aims such as improving equity and ensuring sustainability of health care provision. For example, the rising consumer demands and expectations for high technology and high-quality medical care due to improved standards of living, changing disease patterns and demographic changes, has led to continuous increase of health care costs that is deemed unsustainable.

Universal Health Coverage (UHC) is defined as "all people receiving quality health services that meet their needs without being exposed to financial hardship in paying for the services".[1] The definition of UHC embodies three related objectives[2]:

1. Equity of access to health services— those who need the services should get them, not only those who can pay for them
2. Good quality health services— improving the health of those receiving services
3. Financial risk protection— ensuring that the cost of using care does not put people at risk of financial hardship

Policy makers have been looking into restructuring the national health care financing and the health care delivery system. They realized that sustainability is unlikely to be achieved through incremental changes. Instead, they are seeking transformative solutions that require dialogue and cooperation across industry sectors and governments, which challenge the current boundaries of health care and traditional established norms of operation.

Under the mid-term review of the 11th Malaysia Plan, the strategy for healthcare remains to ensure universal access while improving equity and efficiency, and the quality of life of the population as we aspire to move toward a high-income country. Policymakers and industry are therefore looking for opportunities to drive quality, stretch funding, and slow cost growth in the health care system.

Current Performance Gap

Value and innovation should be recognized and rewarded. While the rest of the health care system is paying for value, procurement for medicines in Malaysia largely continue to be centered in the old construct that focuses solely on price, regardless of the health outcomes of each patient. Consequently, many innovative medicines have not been listed in the past years on the Ministry of Health Medicines Formulary which has led to a high out-of-pocket medical cost affecting Malaysian households in general.[3]

Decisions on listing innovative therapy should reflect the clinical needs and values of the population and its rationale should be made explicit for open dialogue with all relevant stakeholders. Assessments of health technologies should be based on broad value concept taking into account all relevant parties and impact to patient population. Positive decisions should lead to greater access and outcomes.

In today's economic climate, targeting health care expenditure for cost-cutting may seem inevitable as part of broader austerity programs. PhAMA believes however that failure to prioritize value improvement in health care delivery and to measure health outcome could further lead to ill-advised "believed" cost containment, impact performance and affect the true goals of Universal Health Care.[4] It is therefore essential to ensure that short-term priorities do not negatively impact long-term value.

The Pharmaceutical Services Programme (PSP) of the Ministry of Health has incorporated an evidence-based evaluation of new treatments to inform formulary listing decision, although not described as formal "health technology assessment (HTA)", PSP and allied partners have started working on a more rigorous and transparent system based on cost-effectiveness thresholds.

Priority setting is an essential component in order for countries to achieve and maintain UHC, as it helps to provide a comprehensive range of key services that are well-aligned with other social goals concerning health maximisation, health distribution, and financial risk protection. Cost-effectiveness analysis is usually the way to provide the largest possible sum of health benefits for a given budget. However, using cost-effectiveness as the sole or main criterion in priority setting gives rise to ethical issues. Two other criteria, namely priority to the worse-off and financial risk protection, should also be taken into consideration in the process of ethical priority setting. When determining the "value" of a health care intervention, policy makers should reflect on the evidence that point to the inclusion of factors other than cost-effectiveness, such as innovation, unmet need, disease severity, and target population size.

PhAMA applauds policymakers are looking for models abroad, primarily with their public sector/policymakers' peers in neighboring countries like Thailand or Taiwan or more developed countries like the UK, Canada, and Australia. However, attention needs to be paid to the appropriateness of transferring particular models to the Malaysian context.

Challenges involved in implementing cost-effectiveness thresholds

While there is a need to define value in health care, cost-effectiveness thresholds often play a disproportionate role in the assessment (as opposed to other factors such as ethical or social issues), despite their arbitrary nature.[5] Economic considerations are important when it comes to the introduction of new technologies or assessment of existing ones, but they should not be subject to rigid limits that cancel out all other aspects.

The WHO Secretariat believes a fixed cost-effectiveness threshold should not be used as an isolated criterion for decision-making, as noted during the Consultation Workshop on HTA for UHC and Reimbursement System (Geneva, Nov 2015). The report is available from the URL:
http://www.who.int/health-technology-assessment/HTA_November_meeting_report_Final.pdf

Fixed thresholds are not very informative, except perhaps in narrowing the field of options for consideration when used in conjunction with other criteria. Contingent valuation on health states using Willingness to Pay (WTP) method is studied in many countries (usually in developed countries). WTP can be varying upon each contingency such as size of health gain, duration of health gain, type of health gain, health gain for whom, and so on.

According to Shiroiwa et al. (2011, Health Economics), WTP for a family member is much higher than for self in all the countries investigated (Japan, Korea, Taiwan, Australia, UK, US). HTAsiaLink, the regional HTA network, initiated a couple of collaborative research projects on this method, Multi-Criteria Decision Analysis (MCDA). For multiple criteria, a set of pre-set weights are applied to calculate a comprehensive value of intervention.

UK National Institute for Health and Care Excellence (NICE) does not endorse MCDA but they rely on the conventional technology appraisal process. Evaluations conducted by NICE in England and Wales, the country's reimbursement decision-maker, provide the most prominent instance where the systematic application of decision rules based on cost-effectiveness ratios has led to politically unacceptable reimbursement decisions. NICE has set its explicit willingness-to-pay threshold between GBP 20,000 and 30,000 per Quality Adjusted Life Years (QALY). However, many new end-of-life treatments do not meet NICE's criterion of purchasing an additional QALY at £20,000-£30,000. Instead, cost estimates for new renal cancer treatments, for instance, amount to £70,000-£170,000 per QALY. NICE has refused coverage to many innovative oncology medicines on

this basis. This has repeatedly resulted in citizens' demonstrations and public and media outrage which has led the UK government to come up with adjustments (system "fixes") to its strict threshold policy:

NICE's End-of-Life Guidance permits disregard of its cost-per-QALY threshold if the costly treatment is limited to a small, terminally ill patient population and if there is robust evidence that the treatment extends life expectancy by at least three months compared to the current NHS treatment.

NICE recommendations on the use of highly specialized technologies (e.g. rare disease medicines) are made by an independent advisory committee called the Highly Specialized Technologies Evaluation Committee. NICE does not apply formal cost-effectiveness thresholds in this area. This was established to provide its citizens access to new therapies, in order to cope with a limited annual budget.

The NICE model as illustrated above focuses excessively on cost-effectiveness and neglects ethical, social, organizational and other key factors such as societal solidarity, patient preferences or the value of end-of-life treatment.

Health care decision making needs to consider multiple factors including economic values. It is not recommended to rely solely on ICER value to make decisions, especially using a fixed ICER threshold based on GDP. Alternative methods such as MCDA can increase transparency and predictability of decisions. However, we still have a long way to go since it is a new concept. PhAMA and the Pharmaceutical Services Programme (PSP) of the Ministry of Health are collaborating to help accelerate the transition towards a value-based system with policy proposals that will encourage access to high-value medicines and treatments and ensure that patients are getting the best value for their health care.

PhAMA believes measuring value correctly will also permit reform of the reimbursement system so that it rewards value by providing health package covering the full care cycle or, for chronic conditions, covering periods of a year or more. Aligning reimbursement with value in this way rewards innovators for efficiency in achieving good outcomes while creating accountability for substandard care.

Health system leaders need to think for the future, expanding the group of responsible stakeholders and breaking from the status quo to deliver high quality, full-access, affordable, and sustainable health services. However, existing framework, process and regulatory barriers are stifling this innovation.

Value assessment models need to be aligned with patient-centered care

PhAMA believes that a deliberate and comprehensive shift to patient-centered care is needed. All stakeholders need to recognize divergent perspectives on value and center on patient value as defined by patient clinical needs and preferences based on their quality of life. This may be done through:

- Personalizing value measurements, including biologic differences among patients
- Incorporating broader measures of value, including outcomes and quality of life
- Measuring the evolution of value of innovative treatments over time

This shift can only be made possible if there is a continuous learning health care system in place. Thus, PhAMA Policy suggestions to support such a system are to:

- Accelerate the incorporation of research parameters into meaningful use guidelines for electronic health records
- Incentivize the consistent collection of outcomes data in standards-based form and update and maintain registries

- Develop a policy framework that supports the accessibility of clinical data for researchers and incentivizes data sharing
- Incentivize standards-based data collection and data exchange capabilities in research

To address this issue, the public sector should partner with industry to facilitate the development and validation of innovative medicines, as well as to ensure the establishment of reliable supply chains that bring beneficial products to clinical practice. Once a product is developed and tested, the public sector needs to work with industry partners to ensure the product is available and affordable on a population basis. Such public-private partnering is key to the development and translation of innovations in research to clinical care and public health.[1]

Appropriate pricing and reimbursement can help manage health care costs and at the same time encourage innovation in R&D and treatment. To provide incentives for innovation in the pharmaceutical industry and thereby facilitate timely access to innovative drugs, governments could take the following steps[6]:

- Introduce "fast track" market authorization approval and swiftly follow with reimbursement procedures
- Introduce conditional reimbursement and pricing, where access is granted to innovative drugs while "real world" data collection continues
- Ensure reimbursement and pricing policies contain some degree of flexibility, where levels are adjusted as new data become available

Industry proactive approach

- PhAMA proposes the adoption of a model for **managed access** to medicines as a means of addressing concerns regarding evidence gaps and timeliness in the Ministry of Health Medicines Formulary assessment process.
- PhAMA believes that **early entry agreement** should be made available for value-added medicines to allow bringing evidence along commercialisation.
- PhAMA remains committed to providing early access to new medicines for Malaysian patients treated in government hospitals trough **Patient Assistance Programs (PAPs)**, already available for patients in the private sector.

Summary

- While UHC is established in Malaysia, it must continue to drive value for its population.
- Priority setting is an essential component in the path to UHC, but the current emphasis on cost-effectiveness analysis in priority setting leads to inequity, disincentives innovation, and prevents patient access to novel, innovative therapies.
- Public-private partnerships play an integral role in developing and translating innovation in health care, research to clinical care and public health, thereby increasing health care equity.
- Additionally, the implementation of appropriate reimbursement policies to health manages care costs and provides incentives for innovation and treatment.
- Malaysia should also consider the use of MCDA to provide an enhanced network for assessment of value, which can in turn help achieve the goal of maximizing utility and rewarding medicines for the value they create.
- Multi-level engagement, government-innovative industry collaboration and leadership are needed to drive these transformative changes in health care.

References

1. Radu, Ciprian-Paul, and Ruxandra Cernea. Romanian Quick-HTA Development in 2013. Value in Health. 2014; 17.7: A444.
2. Ottersen T, Norheim OF. Making fair choices on the path to universal health coverage. Bulletin of the World Health Organization 2014;92:389-389.
3. Catastrophic health expenditure and 12-month mortality associated with cancer in Southeast Asia: results from a longitudinal study in eight countries, The ACTION Study Group, BMC Medicine (2015) 13:190.
4. What If All Cancer Patients In Malaysia Had Access To The Best Available Care: How Many Deaths Are Avoidable? G.F. Hoa,* et al. European Journal of Cancer Volume 50, Supplement 4, May 2014, Pages e2–e3, 6th Asian Oncology Summit and 10th Annual Conference of the Organisation for Oncology and Translational Research.
5. Akaza H, Roh JK, Hao X, et al. UICC International Session: What are the implications of sharing the concept of Universal Health Coverage for cancer in Asia? Cancer science 2016;107:556-563.
6. Kanavos PS, Richard; Lewison, Grant; Schurer, Willemien; Eckhouse, Seth; Vlachopioti, Zefi. The role of funding and policies on innovation in cancer drug development. 2010.