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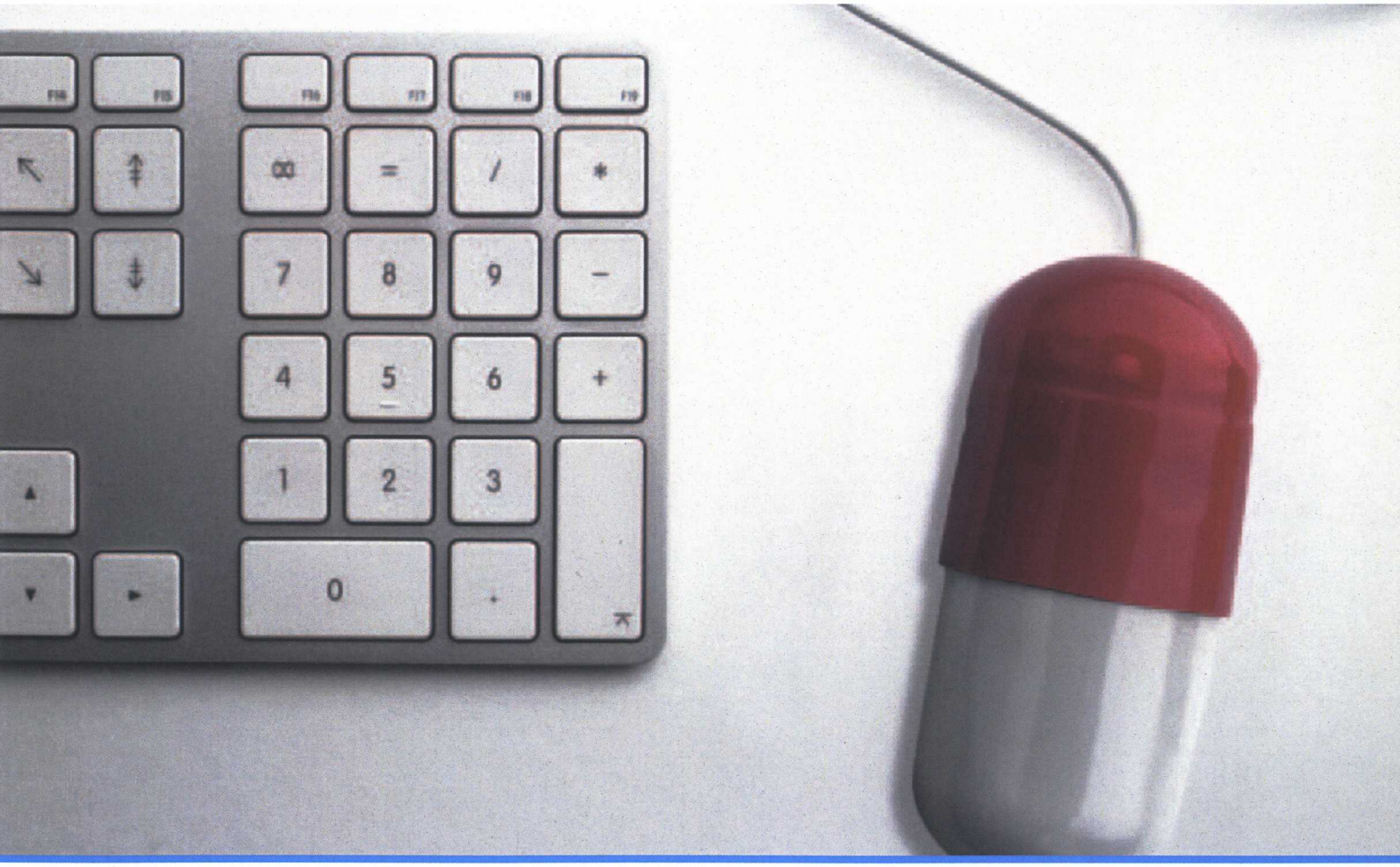
PhAMA
'Innovative Medicines for Malaysia'



Building Greater Access to Innovative Medicines – What is Next for Malaysia?

An analysis of proposed reforms and a review of possible access schemes

Contents

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Abstract

BACKGROUND

This paper takes an in-depth look at an issue of growing significance in Malaysia—patient access to medicines. How should access be managed? What are the challenges facing Malaysia, especially in this era of proposed reforms under the National Medicines Policy, 2nd Edition (2012).

METHODOLOGY

IMS Health, an independent third-party provider of healthcare information, consulting and technology services was commissioned by PhAMA Malaysia to review the National Medicines Policy and examine the ways that countries like South Korea, Taiwan and Japan manage access, by weighing the pros and cons of approaches ranging from clinical effectiveness evaluation alone to clinical effectiveness coupled with budget impact analysis and cost effectiveness combined with budget impact analysis.

Our research included an analysis of the impact of post listing status of medicines in the MoH Drug Formulary on access, as measured by the changes in volume uptake for three key therapeutic areas. We also examined the relative availability of innovative medicines in Malaysia as compared to other Asian countries.
We relied on IMS MIDAS data, particularly the data collected on DPPIV inhibitors for diabetes mellitus (DM) and biological therapies for rheumatoid arthritis (RA).

We have also covered some alternative access schemes, such as risk-sharing agreements and coverage with evidence development that have been implemented in other countries.

KEY FINDINGS

1. After analyzing the 2009-2013 product growth rates in 3 specialty and branded original products in oncology, rheumatoid arthritis and pain in Malaysia's public versus private channels, it was determined that despite MoH Drug Formulary Listing status, access to certain medicines through the public sector remains low or unchanged.

2. Access to publicly-funded innovative medicines such as DPPIV inhibitors and biological therapies is consistently at 0.4%, which is lower compared to Korea and Taiwan. The low access suggests that there are a great number of Type-2 diabetes mellitus and rheumatoid arthritis patients in need than are currently being treated with these therapies.

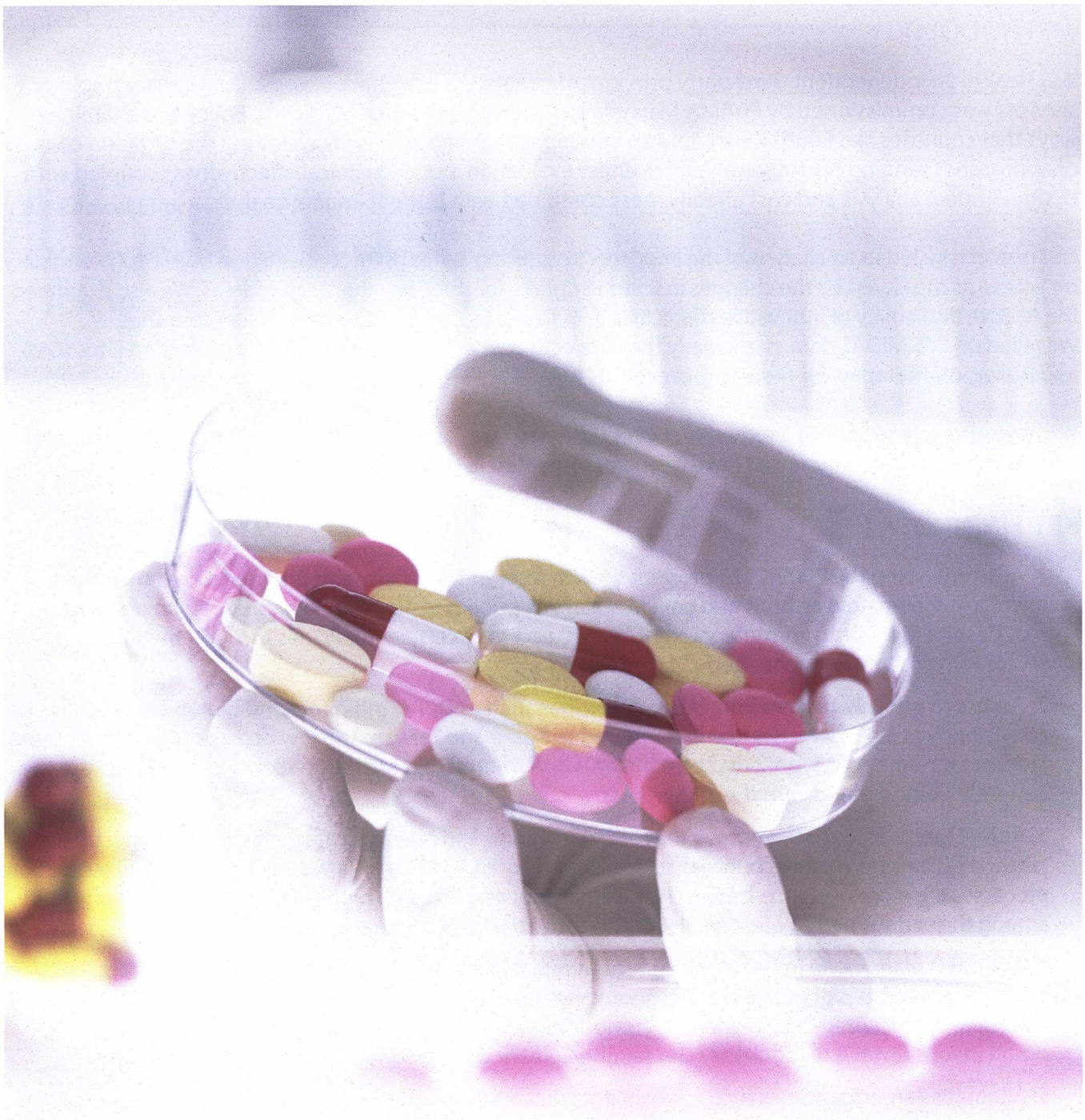
3. As MoH steps forward to improve the formulary listing and financing mechanisms, it is important to take the lessons from other countries such as South Korea, Japan and Taiwan to ensure that all evaluation processes are more evidence-based, clear, transparent and fair, with high-quality communications among the various parties responsible for the evaluation and engagement process.

4. As MoH is planning to improve the current formulary listing and financing mechanisms, access to innovative medicines at public facilities should not be delayed. In many countries, reimbursement application is accepted once marketing authorization is granted for a product. In fact in Australia, simultaneous reviews of regulatory and reimbursement applications are allowed by authorities.

5. Alternative access schemes such as finance- or outcome-based agreements should be further explored and implemented through a dedicated fund to enable wider access to innovative therapies for critically ill patients.

CONCLUSIONS

In the absence of details in the proposed reforms to the National Medicines Policy in Malaysia, it is speculative and difficult to make in-depth recommendations regarding the best next steps. However, in order to realize the vision of universal coverage, it is clear that Malaysia must seek out greater public funding and financing while flexibly exploring different possible access schemes in order to improve and provide sustainable access to innovative medicines.



Abbreviations used in this paper

- ASEAN– The Association of Southeast Asian Nations
- AIFA - The Italian Medicines Agency
- BIA - Budget Impact Analysis
- BNHI - Bureau of National Health Insurance
- BOPC - British Oncology Pharmacy Association
- CDE - Center for Drug Evaluation
- CED - Coverage with Evidence Development
- CFFE - Conditionally Funded Field Evaluation
- Chuikyo - Central Social Insurance Medical Council
- DBC - Drug Benefit Committee
- DDPIV - Dipeptidyl-peptidase IV
- DM - Diabetes mellitus
- DPO - Drug Pricing Organization
- EU - European Union
- GBP - British Pound
- GDP - Gross Domestic Product
- HER2 - Human epidermal growth factor receptor 2
- HIRA - Health Insurance Review & Assessment Service
- HiTAP - Health Intervention and Technology Assessment Program
- HTA - Health Technology Assessment
- ICER - Incremental Cost Effectiveness Ratio
- ISPOR - International Society For Pharmacoeconomics and Outcomes Research
- KW - Korean Won
- MAF - Medication Assistance Fund
- MedPrice - Medicines Price Monitoring System
- MHLW - Ministry of Health Labour and Welfare
- MIDAS - Multinational Integrated Data Analysis System
- MoH - Ministry of Health
- MOHW - Ministry of Health and Welfare
- NHI - National Health Insurance
- NHIA - National Health Insurance Administration
- NHIC - National Health Insurance Cooperation
- NHMS - National Health Morbidity Surveys
- NHS - National Health Service
- NICE - National Institute for Health and Care Excellence
- OECD - Organisation for Economic Co-operation and Development
- PAS - Patient Access Scheme
- PBAC - Pharmaceuticals Benefits Advisory Committee
- PBPC - Pharmaceutical Benefits Pricing Authority
- PBRS - Pharmaceutical Benefit and Price Schedule
- PLS - Positive List System
- PPP - Purchasing Power Parity
- P4P - Pay for Performance
- QALY - Quality-Adjusted Life-Year
- RA - Rheumatoid Arthritis
- RM - Ringgit Malaysia
- SHA - System of Health Accounts
- SHI - Social Health Insurance
- SHIs - Statutory Health Insurance
- TAC - Together Against Cancer
- USA - United States of America
- UK - United Kingdom

Chapter 1: Executive Summary

The discovery and development of new drugs is only one small piece of a global healthcare puzzle. Indeed, ensuring that patients have access to the drugs they need—through rational selection, appropriate pricing, sustainable financing, and well-designed delivery systems—is an equally challenging endeavor.

But how does one manage access, especially during an era in which healthcare budgets are increasingly squeezed by changing demographics, new technologies, and rising expectations? What pathways have other countries taken—to what ultimate result? When do new medicines represent good value, and how does the adoption of a new medicine on a national formulary affect an overall healthcare budget?

Such questions are particularly relevant in Malaysia, where proposed reforms under the National Medicines Policy, 2nd Edition (2012) are moving the country toward a National Formulary. Should the Ministry of Health (MoH) in Malaysia adopt clinical effectiveness alone as its access criteria, or should clinical effectiveness be paired with a budget impact analysis? What would happen, conversely, if Malaysia developed guidelines reflective of cost effectiveness criteria coupled with a budget impact analysis?

In this white paper we take a close look at the new policy while we share our illustrative analysis on the uptake and access of some pharmaceutical products in Malaysia. We delve into common access models and examine other alternative arrangements currently in use in other countries. Finally, we discuss key considerations and potential strategies that might be adopted to improve the access gaps in Malaysia.

In the course of our analysis, we concluded that despite MoH Drug Formulary listing status, access to certain medicines through public channels remains unchanged or low. The annual growth in public sector uptake of an oncology product and a rheumatoid arthritis product was nearly 7.5% and 4.7% between 2009 and 2013, respectively. While the pain product showed better growth at 13% per year, overall its volume sold was higher in the private sector than the public sector.

Another analysis indicates that there might be more patients in need of DPPIV inhibitors and biological therapy for type-2 DM and RA, respectively, than those are currently being treated. We determined that the proportion of patients in Malaysia with access to DPPIV inhibitor therapies is consistently 0.4%, which is lower than in Korea (9.8%) and Taiwan (10.8%). The same was observed for RA, where 0.21% of patients have access to biological therapies, compared to 9.2% in Taiwan and 4.9% in Korea. All the DPPIV inhibitors and biological therapies included in these analyses are covered by the public payers in these countries.

Limited access could be due to reasons such as inaccurate budgeting, insufficient funding or capitation applied on the volume of a product or the number of patients treated with a product in a fixed period of time. Capitation may impede the patients from getting the treatments they need. Lengthy listing evaluation processes should be avoided if the ultimate goal is to ensure that patients are treated with the medicines they need.

In our review, while the formulary listing is moving forward to be more evidence based, a consistent lesson learned again and again from the experiences of other countries such as South Korea, Japan and Taiwan is that clarity, fairness, and transparency in all evaluation processes will be key going forward to improve patient access to innovative medicines. High-quality communications among the various parties responsible for the evaluation and engagement process will also be critical. Indeed, improved communications are a top priority for Malaysia and its MoH as it steps forward with its new programs and policies.

As MoH works to improve the current formulary listing and financing mechanisms, access to innovative medicines at public facilities should not be delayed. In many countries such as the UK, Japan, South Korea and Taiwan, reimbursement application is accepted once marketing authorization is granted for a product. And in fact in Australia, simultaneous reviews of regulatory and reimbursement applications are allowed by authorities.

Besides, alternative access schemes such as finance- or outcome-based agreements should be explored and implemented through a dedicated fund that will enable wider access to innovative therapies for critically ill patients in Malaysia.

In the absence of details in the proposed reforms to the National Medicines Policy in Malaysia, it is therefore both speculative and difficult to make in-depth recommendations regarding best next steps. However, in order to realize the vision of universal coverage, it is clear that Malaysia must seek out greater public funding and financing while flexibly exploring different possible access schemes in order to improve and provide sustainable access to innovative medicines.

Chapter 2: The Changing Healthcare Landscape in Malaysia

Access to medicine, as defined by the World Health Organization (WHO), is a function of four key attributes: the rational selection and use of medicines, affordable pricing, sustainable financing, and reliable health delivery systems[1].

In Malaysia, where a top priority is quality care for an evolving population, society is at a crossroads. Certainly, equitable access to medicines is recognized as fundamentally important. But financial pressures persist.

Against this backdrop, a number of alternate access models have been proposed, each with its own set of pros and cons. To get a better understanding of future possibilities, we begin with a look at the five core components that make up the evolving Malaysian healthcare system.

- Healthcare delivery
- Healthcare **financing**
- Formulary listing
- Medicine availability
- Medicine pricing

HEALTHCARE DELIVERY

Malaysia has a two-tier healthcare system, with the public sector providing about 82% of inpatient care and 35% of ambulatory care, and the private sector accounting for some 18% of inpatient care and 62% of ambulatory care. Personal visits to other providers such as traditional medicines make up the remaining 3% of ambulatory care[2].

Public sector services are highly subsidized, rendering goods and services either free to the user or accessible through small co-payments; in fact, only 2-3% of the MoH expenditure is recouped from patient charges[3]. The MoH provides primary care, secondary care and tertiary care through health facilities such as general hospitals, district hospitals and health clinics.

There are 143 public sector hospitals in Malaysia, 136 of which are MoH hospitals(3). In addition to the MoH, health-related services are provided by the Ministry of Defense, which supports healthcare needs for individuals in the armed forces and their families; the Ministry of National Unity and Social Development, which is focused on aboriginal affairs; and the Ministry of Housing and Local Government, which provides environmental health services. The long-term care of the elderly is supported by the Ministry of Women, Family and Community Development, while drug rehabilitation care is sponsored by the Ministry of Home Affairs[4]. Finally, patients can be referred for partially but heavily subsidized medical care at university hospitals under the Ministry of Higher Education.

Private health providers complement the government-sponsored medical services by offering curative services through general practitioner clinics, medical centres and private hospitals.

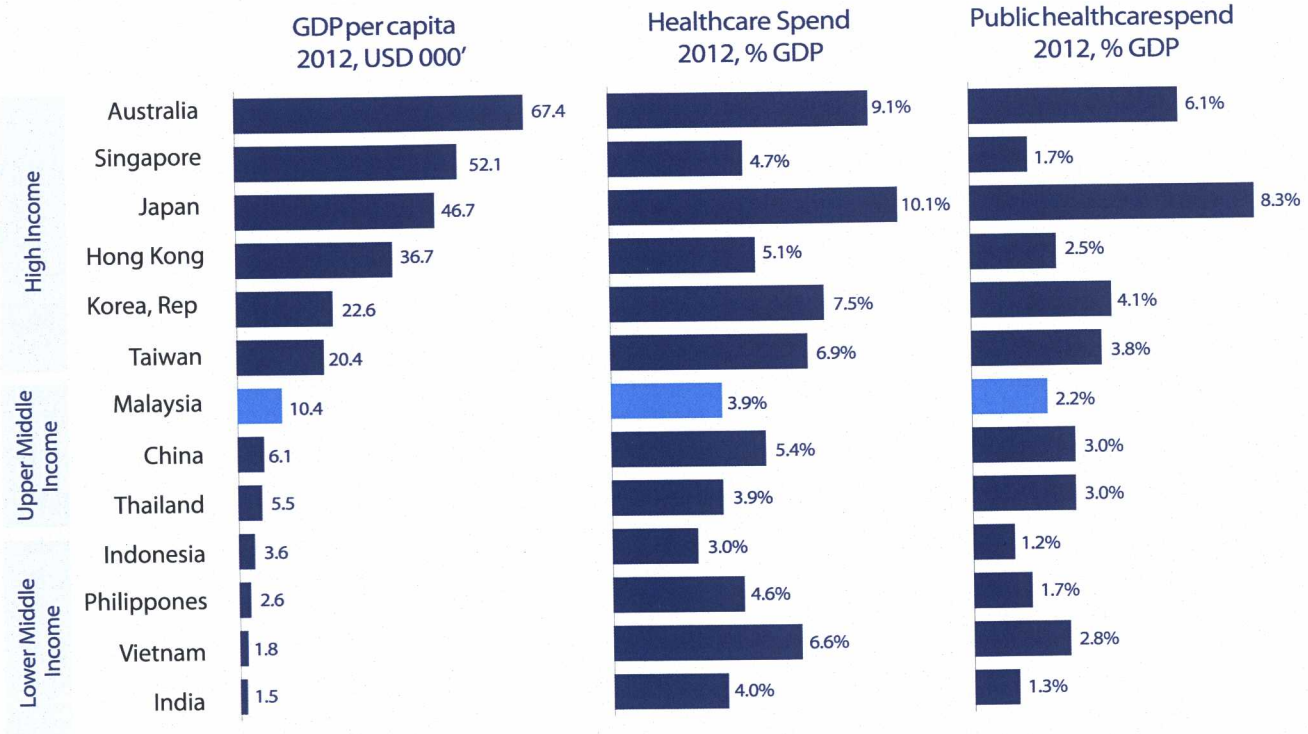
HEALTHCARE FINANCING

The Malaysian public healthcare system is tax-financed. Private healthcare, on the other hand, is largely funded by household out-of-pocket payments, which accounted for 79% of private healthcare expenditure in 2012[5].

In recent times, demographics and lifestyle have created a need for expensive, long-term treatment, placing pressure on public spending. There is, for example, proof that the prevalence of diabetes and hypertension is on the rise, according to the National Health Morbidity Surveys (NHMS) conducted in 1996 (NHMS II), 2006 (NHMS III) and 2011 (NHMS IV). Moreover, the prevalence of Diabetes Mellitus (DM) has increased more than two-fold in NHMS IV (20.8%) as compared to NHMS II (8.3%), and higher prevalence in Hypertension and Cardiovascular Disease has also been observed in NHMS IV[6].

Meanwhile, advances in medical technology and a more empowered patient population are elevating demands for higher-quality healthcare services, further squeezing healthcare funding. According to the WHO Health Financing Strategy for the Asia Pacific Region (2010-2015), total health expenditure should be at least 4-5% of the gross domestic product in order to sustain universal healthcare coverage, while out-of-pocket expenditure should not exceed 30-40% of total healthcare expenditure. Malaysia sits on the borderline of both of these indicators, with SHA-based total healthcare expenditure at 3.9% of GDP in 2012 and out of pocket expenditure at 36% of total healthcare expenditure[7].

Figure 1: Comparison of macroeconomic status of Asia Pacific countries



Source: World Bank 2012 (latest available data), Hong Kong Census and Statistics Department, National Statistics Taiwan

In an analysis comparing Malaysia to a broader set of Asia Pacific markets (including India and Vietnam), IMS Health found that Malaysia has the highest GDP per capita (USD 10.4k) of other upper middle income markets defined by the World Bank, although healthcare spend is lower by comparison [Figure 1]

In terms of public spend on pharmaceuticals, the Malaysian government invested approximately 11.8% of its total public healthcare budget on medicines in 2011[8]. This is significantly lower than the national pharmaceutical expenditure as a percentage of public healthcare spend in other countries, such as Korea (25%) and Taiwan (25%)[9, 10].

FORMULARY LISTING

In the public sector, prescribing is restricted to drugs listed in the MoH Drug Formulary, where both innovative brands and generic products are available. In the latest 2013 MoH Drug Formulary, 1608 items appear on the list, an increase of 14.2% from 1408 items in 2009. To review and update the MoH Drug Formulary periodically, the MoH has a Health Drug List Review Panel consisting of senior consultants and pharmacists from the MoH. The drug review is based on factors such as clinical advantage, best and current treatment options, current and previous usage, prescribing pattern, approved dosage and indication and cost of treatment. This panel also meets two to three times a year to consider proposals for new drugs as well as the deletion, alteration and addition of the drug/dosage[3]. While international or local clinical practice guidelines play a key role in the selection or listing of medicines, availability of medicines at public facilities is driven by budgets at the hospital level (Babar et al. 2007; please refer to Analysis I and II below)[11].

MEDICINE AVAILABILITY

One key impact of strained healthcare financing is that access and availability of medicines in government hospitals has increasingly become an issue, with patients experiencing long waiting times for treatments or sometimes not receiving treatments despite their listing in the MoH Drug Formulary. A local study conducted by Babar et al. (2007) concluded that the availability of medicines was low in the public hospitals even for medicines listed in MoH Drug Formulary. A separate consumer survey by Babar et al. 2003 showed that 37% of patients obtain medicines from private hospitals or clinics and 42% from retail pharmacies, requiring significant out-of-pocket expenditures[12].

ANALYSIS I: MEDICINE UPTAKE POST LISTING IN MOH DRUG FORMULARY

Access tends to improve when a medicine is listed for coverage by a third party. Recently, IMS Health studied the impact on access of the post listing status of medicines in the MoH Drug Formulary as measured by the changes in volume uptake.

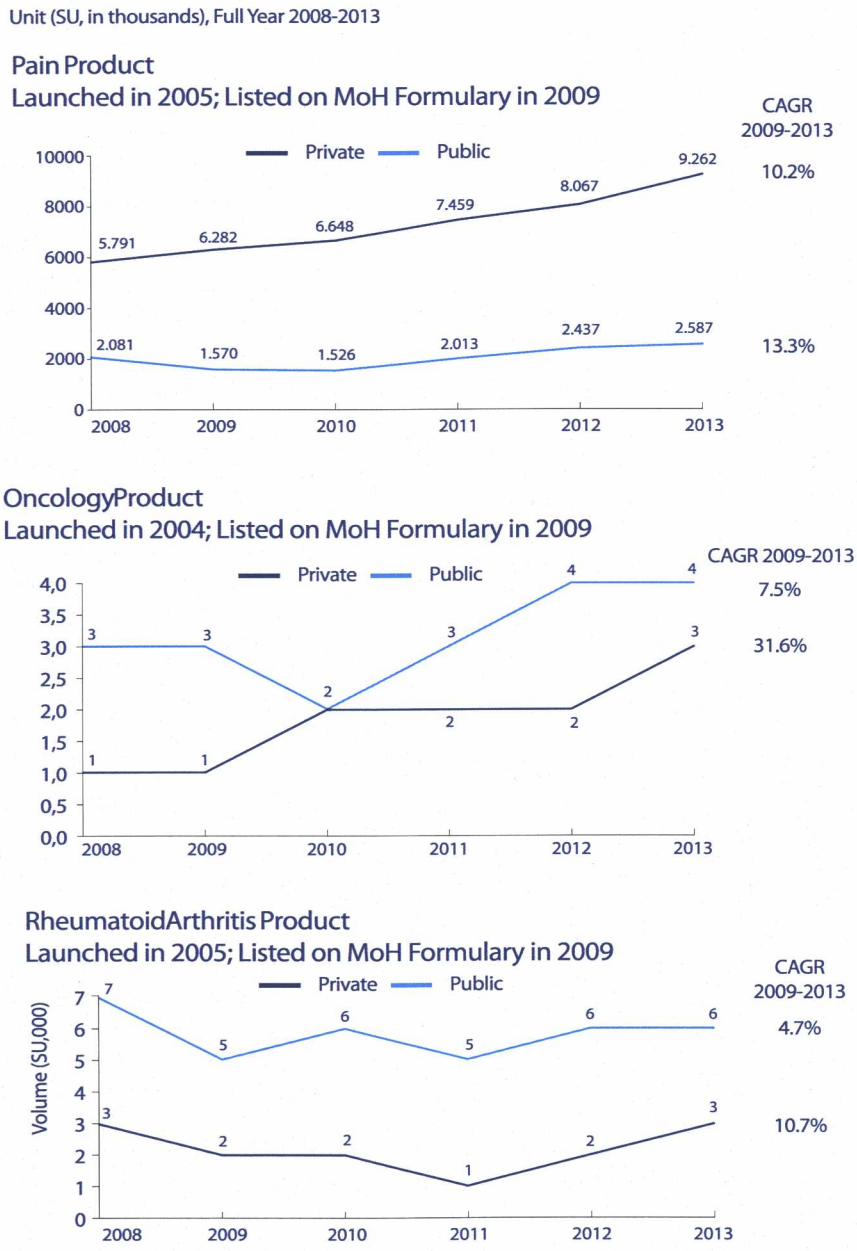
METHODOLOGY

As our starting point we examined the volume sales uptake of three high cost, specialty care and branded original products in oncology, pain and rheumatoid arthritis. Our study covered both the public and private sectors, where we collected and analyzed the relevant IMS MIDAS data and extracted the 2009 MoH Drug Formulary List from the Pharmaceutical Services Division website. Ultimately we identified the listing status of the three branded original medicines of interest.

RESULTS

There was limited growth in the public sector uptake—despite its listing on the MoH Drug Formulary in 2009 [Figure 2] In the case of the oncology product, the growth in public sector uptake between 2009 and 2013 approximated 7.5%, while the rheumatoid arthritis product grew at a rate of 4.7% growth in the same period. The pain product showed better growth at 13%, however, it is overall volume sold was higher in the private sector than the public sector, even in 2013. We concluded that, despite MoH Drug Formulary listing status, access to certain medicines through public channels remains unchanged or low. This observation could be explained by possible capitation exerted on a product's volume or number of patients to be treated by a product. Although it brings budget certainty, capitation based on financial rather than clinical needs and outcomes could harm the choice that physicians have and ultimately access by patients to more clinically appropriate therapies.

Figure 2: Volume uptake of branded original drugs after inclusion in the MoH formulary in 2009



Source: IMS PADDS data, Full year 2008-2014, Unit (SU, in thousands)

ANALYSISII: PROPORTIONOF PATIENTSTREATEDWITH INNOVATIVE MEDICINES

But what about access to new and innovative medicines? True, such access always varies across countries. However, a relatively low access level likely suggests a limited availability of a medicine, especially if we assume fairly standard clinical needs and practice methods.

METHODOLOGY

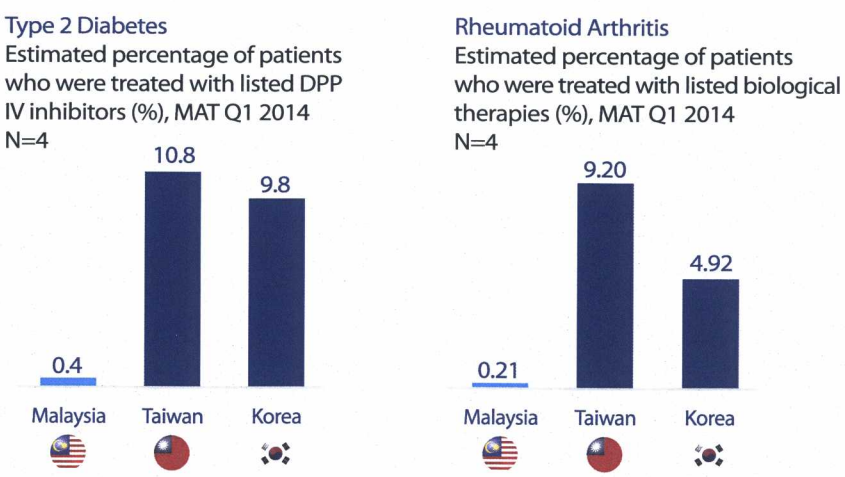
To get to the core of the question, we examined the relative availability of innovative medicines in Malaysia as compared to other Asian countries. We relied on IMS MIDAS data, particularly data collected on DPPIV inhibitors for Diabetes Mellitus (DM) and biological therapies for rheumatoid arthritis (RA)—both relatively new medicines that have been in the local clinical practice for a number of years.

We divided the 2014 volume sales data of each listed DPPIV inhibitor for DM and biological therapy for RA by the corresponding annual dosage estimated using the daily defined dose by WHO to estimate the number of patients treated with these medicines. These numbers were then divided by the estimated total number of patients with Type-2 DM and RA using the reported prevalence rates of these two conditions in the studied countries to derive the proportion of Type-2 DM patients and RA patients treated with DPPIV inhibitors and biological therapies, respectively.

RESULTS

Figure 3illustrates our findings. In short, the proportion of patients in Malaysia with access to DPPIV inhibitor therapies is consistently 0.4%, which is lower than in that of Korea (9.8%) and Taiwan (10.8%). The same was observed for RA, where 0.21% of patients have access to biological therapies, compared to 9.2% in Taiwan and 4.9% in Korea. All the DPPIV inhibitors and biological therapies included in these analyses are covered by the public payers in these countries. The low access findings suggest that there might be more patients in need of DPPIV inhibitors and biological therapy for type-2 DM and RA, respectively, than are currently being treated.

Figure 3: Patient volume analysis



PRICING MEDICINES

Of course, access to treatment is heavily dependent on the availability of affordable medicines. Malaysia has a free pricing system: manufacturers, distributors and retailers are allowed to set their own prices. In the public sector, the prices of medicines are indirectly controlled through tenders and price negotiations[13]. In the private sector, on the other hand, free market competition governs prices, although the Pharmaceutical Services Division has been monitoring private sector prices through the Medicines Price Monitoring System (MedPrice). Medicine pricing, particularly private sector prices, has come under scrutiny more recently due to growing concern regarding the affordability of medicines for middle-to-low-income patients[14].

A separate analysis comparing the prices of medicines in Malaysia and other countries indicates that such costs are not significantly higher in Malaysia than in other countries, even when considering branded medicines in such high-cost therapeutic areas as oncology. When compared to the average prices in other middle income, semi-reimbursed markets such as China, Malaysia's prices were found on average to be approximately 30% lower, although its GDP per capita (current prices, USD) was nearly 54% higher than that of China, while GDP per capita (international prices, PPP) is 80% higher than that of China [7, 15]. (More details on this topic can be gleaned from a separate position paper titled 'Is Reference Pricing Right for Malaysia?')

However, there are challenges ahead as public financing and quality of care struggle to keep pace with population growth, disease and lifestyle trends. As a consequence, availability or access to medicines in the public sector will become increasingly difficult, leading to an affordability challenge for low- and middle-income patients who do not have access to medicines through the public sector and cannot afford private-sector prices. This undermines the fundamental principle upon which Malaysia's healthcare system has been based—that access to quality health care should not depend upon ability to pay.

SEEKING GREATER ACCESS

On August 11, 2009, in response to the growing disparity in the public and private sector services, the MoH proposed the 1Care for 1Malaysia healthcare reform strategy. The point was to restructure the healthcare financing, governance and service delivery in order to make the allocation and expenditure of resources more efficient.

Among other policies, the 1Care for 1Malaysia policy paper outlined new financing mechanisms. Chief among them was a mandatory Social Health Insurance (SHI) contribution, which was to be calculated on a sliding scale as a percentage of income and a governmental contribution (derived from general taxation). This contribution was to cover MoH activities and the SHI premiums for registered poor, disabled, elderly (those over 60), government pensioners and civil servants (+ five dependents).

Unfortunately, the provisional nature of the MoH proposal left it subject to interpretation without specific and relevant details. It soon became an area of controversy among both media and civil society groups and consequently gained little traction[14].

Recent policy measures are narrower in focus. The National Medicines Policy, 2nd Edition, 2012 (Dunas II), for example, aims to address some of the issues of equitable access and affordability through a number of primary strategies, including the development of a unified national drug formulary that seeks one set of standards for the selection and clinical use of medicines[16]. The policy seems to call for the merger of multiple formularies with the MoH Drug Formulary, as well as the creation of a single drug and therapeutic committee. The policy also calls for changes to the selection and listing of medicines based on measures incorporating quality, safety, efficacy, clinical effectiveness and cost effectiveness of the treatment.

Less explicitly, the policy touches upon goals to address the public-private dichotomy in the distribution of healthcare delivery (resource sharing, registered population, providers) and to unify the health financing system (equity in resource allocation, one social health insurance) to achieve access equity.

Finally, the National Medicines Policy seeks to address the affordability issue through mandatory price reporting, monitoring, reference and control mechanisms[16].

(For more information, please refer to a separate position paper entitled "Is Reference Pricing for Malaysia?')

Chapter 3:
Taking a Close Look at Existing Access Models

Given the lack of detail in the proposed Malaysia reforms, it is helpful to take a close look at the approach other countries have taken to this tricky issue of drug access. No country is perfect. Every approach to access has its attendant pros and cons. But every approach teaches important lessons about what is possible and what might be addressed—both by overall policies and by separate schemes.

Broadly speaking there are three models. Each reflects a different perspective on calculating and weighing clinical and cost factors:

- Clinical **effectiveness** evaluation alone
- Clinical **effectiveness** evaluation and budget impact analysis
- Cost **effectiveness** and budge timpact analyses

3.1 CLINICALEFFECTIVENESSEVALUATIONALONE

This model focuses on clinical value as the key decision-making criterion for drug reimbursement or listing. It compares the new drug versus other available options, usually the standard of care, and assesses whether the drug has innovative or improved clinical usefulness. Efficacy and safety often play the most critical roles in assessing the level of innovation and clinical usefulness of the new drug. Reimbursement pricing determination is the final step of the medicine availability assessment.

To determine the price of a new product, a comparative approach is taken in which the price is set based on the price of a comparator. In cases where a drug has no active comparator, the price is determined by a series of rules that take into account of factors such as costs of overall production and marketing[17]. International reference pricing is also sometimes used as a tool to decide or support pricing decision-making.

(For further information, please refer the other paper on 'Is Reference Pricing Right for Malaysia?')

CASESTUDY1: JAPAN

In Japan, which has a public and compulsory health insurance system, medicines must be listed in the National Health Insurance (NHI) reimbursement price list before they can be accessed. Manufacturers seeking reimbursement for a new drug must submit a combined pricing and reimbursement application to the Ministry of Health, Labour and Welfare (MHLW). New drugs are not eligible for reimbursement until they have been on the NHI reimbursement price list[17].

The manufacturer's combined pricing and reimbursement application must contain the information of the manufacturer's proposed price and the information justifying the calculation of the price. Criteria are established for selecting a valid comparator for evaluation[17]. If a comparator is available in Japan, the application must include information on the comparator, as well as on the proposed price premium, i.e. a higher price than the comparator. Manufacturers also need to submit the drug's price in other markets, particularly the price in France, Germany, the UK and the US. The anticipated patient population size to be treated with the new drug as well as sales forecast for the first 10 years are required[17]. The submission of pharmacoeconomic data is not mandatory in Japan and as such few manufacturers submit such data to support the application[18].

The Drug Pricing Organisation (DPO) reviews submissions, taking into account the presence and suitability of the comparator, the applicability of the price premiums and other cost data. The DPO proposes a price either based on the comparative price method where a comparator is available or cost calculation method in the absence of a valid comparator. Manufacturers are permitted to appeal the proposed price[17].

When a drug displays an increased therapeutic usefulness versus the comparator, it can be granted a price premium in Japan. The criteria are summarized below:

CATEGORY & CRITERIA	PREMIUM
INNOVATIVE Drugs meeting all of the following criteria: • New, clinically useful mechanism of action • Objectively proven to be significantly more effective or safer than existing drugs in the same category • Objectively shown to improve the treatment of the condition indicated	70-120%
USEFULNESS I Drugs meeting two of the above criteria.	35-60%
USEFULNESS II Drugs meeting any one of the above criteria, or which have been shown to offer greater therapeutic benefits than existing drugs in the same category	5-30%
NO IMPROVEMENT	0%

Source: IMS PharmaQuery: Japan: Pricing – Prescription Drugs. June 2014.

Price premium over the comparator may also be granted for drugs that are indicated for a paediatric condition or target a particularly small therapy area. However, according to Kamae, the premium price is decided without scientific evidence at the current stage[19]. Kamae pointed out several accompanying problems, including adjustments made according to political and subjective opinions, lack of academic inputs and the purpose of the price premium mechanism seemingly having been used as a means of cost reduction[19].

Beyond all this, the current system allows for regular price revisions every two years, market expansion price revisions (i.e. price reduction after a drug has broadly penetrated the market) and a 4-6% price cut once generic drugs emerge[20]. Based on this pricing system, Uehara has noted the tendency for drugs to be priced relatively lower in Japan compared to USA and that prices can be cut even during the patent period, which makes Japan's pharmaceutical market unattractive from the industry's perspective[20].

As of 1 April 2014, new drugs may be eligible for the "precursor" premium if they have been marketed in Japan ahead of other countries[17]. At the same time, a new points-based system has been introduced to help calculate these premiums using 19 separate criteria following complaints that the methodology for allocating price premiums lacked transparency[21]. However, the details of these criteria have not yet been disclosed by MHLW or Central Social Insurance Medical Council (Chuikyo). Therefore, it is too early to conclude how these new incentives will have an impact on drug access in Japan.

On the other hand, Japan recognized the key role of pharmacoeconomic studies. In fact, in the 1990s, the MHLW recommended appending pharmacoeconomic data to the dossier for new drug listing applications. Nevertheless, despite the early recognition of the value of economic evaluation, research and education in the field has not sufficiently matured to ensure that cost-effectiveness information is used in healthcare decisions. Compared to the US and EU, research activities on economic evaluations are still very limited in Japan[22]. Manufacturers have not been sufficiently motivated to regularly submit cost-effectiveness data. Still, the value of pharmacoeconomic studies has recently been reaffirmed, with the MHLW and Chuikyo planning a trial implementation of cost-effectiveness evaluation into health services in the near future[23].

Discussions on a standardized methodology are in the works with an aim to improve transparency of appraisal, scientific validity and reproducibility of the cost-effectiveness evaluation[24].

3.2 CLINICAL EFFECTIVENESS EVALUATION AND BUDGET IMPACT ANALYSIS

In this model, both clinical value and budget impact play a role in drug reimbursement. Manufacturers are asked to provide budget impact data when submitting the applications for reimbursement. Similar to the first model (clinical effectiveness evaluation alone), a new drug is compared to the current standard of care in terms of clinical usefulness, with efficacy and safety being the most important drivers of the final decision. Additionally, a budget impact analysis (BIA) estimating the fiscal consequences of adopting the new drug within a specific health context are assessed.

Other decision drivers include the disease severity that the drug is indicated for, the importance of the drug in local treatment guidelines and clinical practice, usage conditions (therapy length, doses per treatment session/ cycle, target criteria, etc.) and benefits in terms of public health (e.g. reduction in mortality/morbidity or disability linked to the disease)[25].

Meanwhile, economic considerations including the total additional therapy costs and other associated costs estimated for a patient population size receiving the new drug with a time horizon (usually 5 years) are evaluated against a pre-determined cap or total budget available for drugs[26]. Often, the listing status, prices and consumption data in other countries are application requirements[25].

Listing decisions tend to be dependent on the reimbursement price that has been negotiated by the manufacturer and decision-making authority based on the medical benefits ratings, prices of comparators, the outcome of health economic evaluation and sales volume forecast and conditions of use[27]. Public health needs, budgetary constraints and market conditions (i.e. the financial impact of the price on health insurance spending and/or spending growth for the corresponding drug class) are taken into consideration[28]. In countries such as Italy and Taiwan, clear rules are established regarding the use of international reference pricing[27, 29].

CASE STUDY 2: TAIWAN

Established in 1995, Taiwan's healthcare system is based on a compulsory National Health Insurance (NHI) scheme that provides virtually universal coverage of 99% of its population(30). Reimbursement of new drugs is managed by the National Health Insurance Agency (NHIA). After receiving marketing approval, a manufacturer can submit application for reimbursement and price approval. All new drug submissions are assessed by the Center for Drug Evaluation (CDE), which provides an assessment report to the NHIA.
The application dossier includes the following key items[31]:

- Basic regulatory information of the drug
- Local disease burden, mortality, prevalence and other epidemiology data
- Comparator information
- **Efficacy** and safety data from clinical trials and other relevant studies
- Budget impact analysis and relevant information such as:
- Reference prices of 10 advanced countries of the product
- Reference prices of products from same category (comparators)
- Cost analysis of the product by estimating the following (reference data is needed):
- The potential target population
- The monthly amount of usage per capita
- The monthly cost per capita
- The monthly total cost
- The annual total amount of usage
- The annual total cost

Since 2014, the NHIA has allowed the products listed in the previous five years to be considered as comparators in the pricing determination process(32). In addition, associated resource use and costs other than drug acquisition costs are to be required by NHIA from 2015 in the budget impact analysis that is mandated in the listing submission dossier. This is in line with the budget impact analysis good practice recommended by the ISPOR Task Force(26). However, transparency remains an issue within NHIA. Unlike the technical assessment reports that are made publicly available by NICE, the technical reports made by Taiwan's CDE are not accessible to externals including the manufacturers whose medicines are being evaluated. Manufacturers are informed of the final decision and there is an appeal process for rejection cases or cases where the decision is not accepted by manufacturers. It is possible to re-submit the dossier to NHIA if new data impacting the comparative or cost effectiveness of the product becomes available[31].

According to The Ministry of Health and Welfare, Taiwan's approach has earned the affirmation of manufacturers, medical service providers, the NHIA and the public by focusing on the assessment quality, efficiency and stakeholder satisfaction[33]. However, manufacturers are not fully satisfied with the pricing mechanism and the speed and transparency of the review process. In the 2013 Position Papers of European Chamber of Commerce Taipei, European manufacturers asked for a more sustainable drug expenditure management, more transparency of the review process and greater speed in providing access to innovative drugs[34].

The assessment process was also criticized for its lack of clear standards and transparency and for its exclusion of some key stakeholders such as patients, physicians and manufacturers[35]. In response, the new process since the introduction of second generation NHI system in 2013, entails a Pharmaceutical Benefit and Price Schedule (PBRS) Stakeholders' meeting that includes NHIA, medical professional professionals and public representatives in decisions regarding new drug reimbursement listing and pricing(36). However, there are some concerns that the new system, which requires consensus at both the expert meeting within NHIA and stakeholders' meeting and includes the possibility of re-review, could delay access to new medications[36]. The current decision making is also criticized for putting too much emphasis on budget impact[35].

Experts believe that it should include cost-effectiveness analyses and local market research so as to better understand the local treatment pattern, unmet needs and epidemiology data[37]. On the other hand, some scholars believe that, although government-appointed bodies should produce the evidence to inform decision making, limited human resources have restricted the ability to do so[38].

Such limitations in capacity should also be considered before applying cost-effectiveness analyses as key criteria for reimbursement[38]. In addition, the fact that local data sources and epidemiologic databases are in short supply presents a challenge not only to Taiwan, but elsewhere throughout Asia[39].

Still, without compromising assessment quality, Taiwan performs economic evaluations at a relatively low cost, on an annual budget equivalent of only 20% and 4% of the yearly funding of its Canadian and UK counterparts, respectively[33]. Taiwan's experience has shown that good results can be produced on an annual budget that is significantly lower than those found in many western countries. In this respect, Taiwan's experience can serve as a good model for middle-income countries[33].

3.3 COSTEFFECTIVENESSANDBUDGETIMPACTANALYSES

In this model, basic clinical information and budget impact analysis are supplemented by cost effectiveness analysis in the assessment of new drugs for reimbursement. Cost effectiveness studies are included in reimbursement and pricing submissions in countries such as the UK, Australia and South Korea.

Cost-effectiveness analysis compares the costs and health effects of a product to assess the extent to which the drug can be regarded as providing value for money against the standard of care[40]. To qualify for reimbursement, the drug typically needs to demonstrate not only clinical effectiveness, but also acceptable cost-effectiveness relative to comparators. Quality-adjusted life-year (QALY) is commonly used for cost-effectiveness analysis.

In addition to the clinical value and the population-based budget impact of a new drug in the previously described model, the cost-effectiveness result will be interpreted against a willingness-to-pay threshold that is not made explicit by the authorities[41]. The cost-effectiveness analysis is always conducted from the payer perspective; the societal perspective that reflects productivity loss is also, at times, considered[42]. The perspective of the analysis dictates the data that are assessed. For instance, the co-payment by patients and non-reimbursement items will not be relevant in the analysis from the payer perspective[43]. Reimbursement status and listed price in other countries are always referenced in the technical evaluation and decision making (see the separate paper on reference pricing).

In countries such as Australia and South Korea, pricing of reimbursed or listed drugs is usually determined through negotiation between manufacturers and payers. Local market comparators as well as international reference pricing are also used. In countries such as South Korea, cost-effectiveness is a key evaluation criterion by Health Insurance Review & Assessment Service (HIRA), which recommends a reimbursement price to be negotiated and agreed upon by NHIC and the manufacturer[44]. And in Australia since January 2011, manufacturers have been able to apply for reimbursement as soon as an application for marketing authorization has been submitted to the Therapeutic Goods Administration (TGA). However, a full marketing authorization must be obtained from the TGA before PBS listing can be finalized[45]. This practice is similar to those in many other countries including the UK, Japan, South Korea and Taiwan. To further improve the access to medicines by patients in Australia, beginning in April 2014, the Pharmaceutical Benefits Pricing Authority (PBPC) ceased in a streamlined process of reimbursement and pricing determination that aims at reducing the time taken to list products on the Pharmaceutical Benefits Scheme (PBS) and improving access to medicines[46].

CASE STUDY 3: SOUTH KOREA

In 2009, healthcare expenditure in South Korea was almost three times the total expenditure for 2001—a situation that escalated the need to demonstrate the value of new medical technologies[47]. In response, the South Korea government introduced the Positive List System (PLS) in 2007, with the intent of maximizing the cost-effectiveness of insured drugs. To be selected for the PLS, drugs need to be effective in both clinical and economic aspects. HIRA is responsible for reimbursement assessment while the National Health Insurance Corporation (NHIC) conducts price negotiations with manufacturers based on HIRA's evaluation result[47]. Manufacturers must submit the following documents for new drugs[44]:

- Productlicense
- Productdescription
- An outline of therapeutic **benefit**
- Clinicaldata and any informationin pharmacopoeia
- Informationpublishedin **scientific** journals
- A comparison with other alternatives (always standard of care)
- A pharmacoeconomic evaluation
  - Cost-effectiveness analysis in terms of incremental cost-effectiveness ratio (ICER) should be the primary method of analysis
  - Information about the comparator is required. However, if there is no appropriate comparator on the market, other treatment (including surgery or best supportive care) may be used for comparison.
- A budget impact analysis to demonstrate the impact on health insurance over a 3-5 year period
- The **current** reimbursement status and listed price of the same drug in foreign countries (Reference to 34 OECD countries as well as Singapore and Taiwan).

Pharmacoeconomic data are not required for orphan drugs, or for "low-priced drugs with the same or improved efficacy" when compared with established reimbursed products[44]. HIRA does not have an explicit cost-effectiveness threshold but refers to GDP per capita, considering it in regard to disease severity, societal burden, quality of life and innovations[47]. However, in general, thresholds of KW 25 to 30 million were used in the published cost-effectiveness studies in the South Korean context[48, 49].

Once a maximum reimbursement price has been recommended by HIRA, the prices of new products are determined through negotiations with NHIC. For innovative new products, prices in OECD countries may be referenced during the pricing negotiations[50]. Reimbursement restrictive to certain patient sub-groups might be the final listing decision in cases where cost-effectiveness value of the new drug is only demonstrated in that particular group of patient population[50].

Price-volume agreements are required for all products seeking reimbursement, except for certain products such as drugs with a reimbursement price lower than the average for the active ingredient, drugs deemed to be essential and others[50]. Negotiations focus on significant differences between the manufacturer's five-year projections and established claims trends for the comparators[50]. The price-volume agreement forms the basis of a contract geared to the initial expenditure on the new drug.

The Korean model is not without issues either. It has been criticized, for example, for the extra financial burden it places on the manufacturers; the submission of economic evaluation data takes time, costs money and can prolong the evaluation period[22].

This two-stage decision-making model, moreover, can further delay patient access to new medications. In the first stage, HIRA conducts the assessment and appraisal process and makes reimbursement decisions. Prices are then negotiated in the second stage by NHIC based on HIRA's evaluation—a process that can delay the time to market entry. These delays are even greater for new chemical entities than for incrementally modified drugs[36].

There are also concerns about how to bridge the gap between the ICER value for pharmacoeconomic studies and decision making[51]. The fundamental problem is the lack of an official definition of an explicit ICER threshold for decision making. Even if there were such a threshold, it would be difficult to connect it directly to final decisions given prevailing uncertainties and limits in data sources[51].

Additionally, HIRA's cost-effectiveness evaluation has been criticized for tremendous uncertainties due to factors such as lack of local clinical and other data, not to mention unreasonable model assumptions that sometimes do not reflect real clinical practice[52]. In addition, clinical trial evidence that ignores the differences in demographic characteristics of the population, treatment pattern and difference in the use of comparators were found to be difficult to apply in the real world[53].

Finally, Korea's duplication of administration between HIRA and NHIC has created challenges for decision makers who must choose between contradicting cost-effectiveness and budget impact analyses[52]. As mentioned above, HIRA decides the initial price of a new drug based on ICER results. Later, drug prices are negotiated and finalized at NHIC, which then utilizes budget impact analyses to determine affordability[52]. Potentially there may be circumstances where the cost-effectiveness analysis indicates an efficient technology while the budget impact analysis results suggest the opposite[54]. Unfortunately, there is no current scientific guidance on how to resolve this dilemma[54].

And yet, the issues notwithstanding, the Korean model has been very successful in controlling healthcare expenditure. After PLS was implemented, the annual growth rate of drug expenditure in South Korea decreased from 14.5% during 2001 and 2005 to 11.5% during 2007 and 2009[47].

HTA: A Short Summary of Its Implementation Challenges

Clearly HTA has the potential to assist payers in making evidence-based decisions about the coverage of medications. It is possible, however, that a poorly designed or managed HTA process runs the risk of denying patients appropriate access to innovative medical technologies, inefficiently allocating resources, constraining clinical freedom and sending distorted signals to medical technology providers[55].
It is therefore important that key HTA principles, as defined by The International Group for HTA Advancement are followed and that the system be designed to capture the full value of medication[56]. In addition, it is important to have adequate evidence about cost-effectiveness, among other things, as one of the bases for treatment recommendations. It is equally important, however, that requirements for evidence do not delay patient access to new drug therapies. There is thus a trade-off between access to reliable evidence and a fast uptake of new, effective, treatments.

To implement an HTA system relying on cost-effectiveness analysis, an explicit threshold promotes informed, transparent and consistent reimbursement decision making. However, controversies remain regarding the appropriate value of thresholds that may be heavily contingent upon epidemiological, medical, political, ethical, cultural, budgetary, elicitation methodology and other factors, and therefore likely to vary across time and space. But in practice, the threshold value is usually chosen arbitrarily. In view of this, the threshold is not a strict decision-making criterion, and trade-offs between cost-effectiveness and other important decision-making factors are usually relevant [57].

Chapter 4:
Other Access Design Considerations and Schemes

Ultimately, two fundamental questions must be addressed in the conversation regarding reimbursement:

- Is a new medicine good value for money?
- How does the adoption of a new medicine ***affect*** the healthcare budget?

These questions could be systematically and independently addressed through health technology assessments by dedicated agencies such as HIRA in South Korea, CDE in Taiwan and HiTAP in Thailand.

Reports produced by HTA agencies supporting decision-making, which aim at improving the quality and cost-effectiveness of the use of health technologies, can be expected to have a strong influence on patient access to these technologies[58]. While HTA and economic evaluations are helpful to assess the value of new drug therapies in relation to their costs, the allocation of appropriate financial resources is a real issue. In addition, evidence beyond that was collected in registration studies is rarely available at the time of a new drug's launch.

Nevertheless, authorities are expected to make reimbursement and pricing decisions without adequate information. In the case of cancer, the gains in life expectancy always come at a very high price. Despite the amount of HTA activity, the ability of cancer patients to access new innovative cancer drugs in countries such as the UK lags behind other countries[58]. Similarly, governments in many other countries are trying to cope with this challenge of balancing entitlement to the best possible care against the financial sustainability of healthcare systems.

Our analysis as shown in Figure 3suggests low patient access despite having innovative medicines such as DPPIV inhibitors and biological therapies listed in the MoH Drug Formulary in comparison to those estimated in Taiwan and South Korea. This finding is consistent with the evidence demonstrated in the local study by Babar et al. (2007), which concluded that the availability of medicines was low in the public hospitals even for medicines listed in the MoH Drug Formulary[11]. To achieve universal healthcare coverage as envisaged under 1Care for 1Malaysia, Ng CW et al. (2014) suggests that the country will need to invest more in the public healthcare system than it currently does[59]. Overseas experience suggests that, other than having established listing evaluation mechanism and sufficient funding, proper health financing models and budget management with payment and allocation flexibility could enable better and more timely access to new medicines[58, 60]. Other practices have been implemented around the world to help manage the funding and accessibility of new medicines.

RESTRICTIVE ACCESS

Authorities can, for example, limit the expenses of a particular drug by restricting the indication for the use of expensive treatments. A new drug can be recommended as part of a stepped or last-line therapy when no other treatment has helped. Alternatively, access can be limited to highly targeted patient groups for which there is clear evidence of comparative clinical effectiveness. Herceptin®, which is particularly effective in HER2-positive patients, is a good example. Payers have also made reimbursement contingent on a positive biomarker test limiting the access to eligible patient populations through which the product value is significantly demonstrated. However, a biomarker that might be used to identify the subgroup of patients that will most benefit from therapies is not readily available for many high-cost products.

CATEGORIZED PRESCRIBING

In many countries, drugs listed in a formulary could be further categorized by different levels of prescriber authorization. This mechanism is already applied in many countries, including Malaysia, in order to control and optimize drug use[61]. Similar strategies, including prior authorization, are required for physicians to prescribe certain listed drugs in order to promote appropriate drug use and hence contain drug cost. However, in response to the changes in local clinical needs, the prescribing categories of the listed drugs need to be routinely reviewed and sometimes revised. Similarly, using generic products that are less expensive than chemically equivalent brand products is an established and widely implemented cost-containment tool[62]. It's important to note that only high-quality products should be made available because low-quality or counterfeit medicines not only make prescribers reluctant to prescribe generic medicines but also causes the public to lose confidence in the entire healthcare system[63-65].

FINANCE-BASED ACCESS AGREEMENTS

Governments have attempted to utilize HTAs to ensure that rational and fair decisions are made regarding resource allocation. Often, however, such attempts have not been deemed cost effective by HTAs such as NICE and have met widespread public and professional discontent[66]. These difficulties are exacerbated when the same drugs that have been refused in the UK are widely available in the US and Europe.

As a result, most countries have some form of access agreement at the population- or patient-level for high-value drugs. Sometimes these are financial agreements in which rebates have been offered to third-party payers for the cost of increased expenditure over an annual subsidization cap, or performance- or outcome-based agreements[67]. In general, the agreements offer a patient-access scheme that seeks to reduce the cost of the drug to payers while also ensuring that patients can gain access to high-cost medicines that might not be deemed cost-effective by payers[68].

In many countries, such as Australia, South Korea and Taiwan, price-volume agreements have been used at the population level. In this case, the number of patients who will receive the therapy is agreed to in advance and rebates in cash or supply of products apply when the total annual expenditure on a treatment exceeds the cap that has been negotiated between the government and the manufacturer. Information such as level of discount, cap, rebates and the like is always held in confidence between the payers and manufacturers. Capitation can be agreed to at patient level in which the therapy is free (or discounted) beyond a specified number of doses or cumulative cost per patient.

Alternatively, free treatment initiation can be offered as another patient-level finance based-access scheme. Examples include the free supply of ranibizumab to NHS for patients with macular degeneration who require the therapy beyond 14 injections[69]. Likewise, the cost of the first three months of certolizumab is covered by the manufacturer to initiate therapy for patients with rheumatoid arthritis in the UK[70].
In South Korea, meanwhile, there are plans to introduce the concept of a "semi-essential drug" that satisfies at least two of the four requirements for "essential" drugs. A "semi-essential" drug will be eligible for the risk-sharing agreement scheme[71]. Clofarabine, a drug usually given to treat children with acute lymphoblastic leukemia, was, for example, selected as a candidate for the risk-sharing agreement scheme with negotiations commencing between manufacturer and MOHW, the NHIC and HIRA[71].

PERFORMANCE BASED ACCESS AGREEMENTS

In performance-based access agreements, which are usually permanent, risk-shifting of guaranteed outcomes or insurance is applied on a per-patient basis. The underlying concept is to avoid inefficient expenditure on treating patients who do not respond to a drug and who cannot be identified ex ante by linking the payment to a drug's performance, or pay-back to non-performance in individual patients[68]. Other terminologies for these agreements include risk-sharing, pay for performance and payback for

non-performance. Schemes under these agreements are managed on a per-patient basis without further real-world data collection and evaluation. Such schemes minimize the possibility of financing a technology that is not cost-effective and therefore improve the payer's willingness to finance the drug[72].

Such an agreement does not lead to more evidence-based reimbursement decision making as compared to schemes under coverage with evidence development. One of the well known cited examples is the repayment of the cost of treating non-responders after four cycles of bortezomib in the UK[73]. Numerous additional examples can be found in the public domain of countries such as France, Sweden and Italy. In the US, unusual access agreements include qualifying patients (non-comorbid, no prior fractures, etc.) with osteoporosis who have taken Actonel® (risedronate sodium) for six out of the nine most recent months; any health costs associated with a nonspinal fracture will be covered by the manufacturing companies[73]. Some academics argue that such contracts are implemented when the payer perceives high risk related to paying for a medicine (it is uncertain that financing a costly drug is good value for money), and the manufacturer is confident that the product has good efficacy (value)[74]. Currently such schemes are being explored between MoH and some multinational pharmaceutical companies to improve patient access to innovative high cost therapies in Malaysia.

COVERAGEWITH EVIDENCEDEVELOPMENT(CED)

Coverage with Evidence Development is a form of conditional reimbursement that is characterized by restricted coverage occurring in parallel with targeted data collection where the stated goal is to reduce material uncertainty. Therefore the access is a temporary/provisional arrangement until new evidence from a cohort of patients over a defined test period facilitates a final decision. CED aims to address the uncertainty related to the decision to finance a medicine. It always leads to a scheduled reassessment of the drug's (cost-) effectiveness, price revision, reimbursement and status condition.

CED additionally goes by the names of "Access with Evidence Development," "Conditional Coverage/ Reimbursement," "Outcome guarantee," "Conditionally Funded Field Evaluation", and "Monitored Use", among others. Examples of pre-specified health outcomes for further evidence collection, analysis and interpretation include real-life effectiveness, higher efficacy in pre-specified subgroups of patient populations, long-term efficacy and safety, improved patient adherence and reduced healthcare resource use. Despite the fact that CED has been increasingly applied for both the reimbursements of new pharmaceuticals and devices in many countries such as US, Canada, Australia, UK, Italy, France, Sweden and Spain, complex political, financial and ethical issues remain[68, 75, 76].

It should be noted that combining different access schemes such as CED and pay-for-performance is a possibility. In Italy, CRONOS project launched by AIFA in Italy to evaluate the real-life effectiveness of Alzheimer's disease drugs (donepezil, rivastigmine and galantamine) collected and analyzed well-defined health outcomes from a cohort of patients. The project studied a nationally representative sample of patients with Alzheimer's disease over a period of two years. In the study, the public insurer reimbursed medicines only in patients who responded at four months of treatment (while the cost for non-responders was covered by manufacturers)[77, 78].

Meanwhile, in the case of the diabetes drug, Januvia® (sitagliptin), an unusual CED arrangement was created in the US between the manufacturer and payers. Rather than getting paid more for good results, discounts were granted to providers if more patients diligently took the drugs as prescribed and if patients' blood sugar was better controlled—regardless of whether the improvement came through Januvia or other medications. This arrangement underlies the mutual perception that patients who take their pills are likely to have fewer complications from the disease and will therefore make fewer claims while lower prices are expected to be offset by an increase in sales volume[73, 79].

END-OF-LIFE DRUG POLICIES

In response to public pressure for a more generous coverage policy for terminal cancer treatments, the UK government has introduced a number of exceptions to NICE's rules[80]. NICE's End-of-Life Guidance permits the violation of its cost-effectiveness threshold if the costly treatment is limited to a small, terminally ill patient population and if there is robust evidence for the treatment to extend life expectancy by at least three months compared to the current NHS treatment. In Australia, "rule of rescue" permits recommend costly but effective medicines for rarely occurring serious or fatal diseases for which no other treatments exist. With PBAC's rather generous assessment standards, the rule of rescue is, however, rarely applied[80].

SEPARATE FUNDING

In some countries (such as France and Germany), separate lists of innovative drugs exist. These lists may include special funding for drugs provided outside of the hospital systems or may enable hospitals to switch to innovative drugs within the restrictions of their hospital budgets. In the UK, a dedicated fund worth GBP 200 million per year, Cancer Drugs Fund, financed separately from the NHS, aims to pay for cancer drugs that have not been approved by NICE and are not available within the NHS[80]. Meanwhile, in Hong Kong, a government fund called 'Samaritan Fund' was established in 1950 to partially subsidize the costs of selected self-financed drugs including certain new cancer therapies for eligible patients through a clear set of criteria and rules in financial assessments[81]. Similarly in Singapore, the Medication Assistance Fund (MAF) was set up by the government in August 2010 to assist eligible means-tested patients on a case-by-case basis to pay for non-subsidized drugs that are not in the Standard Drug List but have been determined to be clinically necessary.

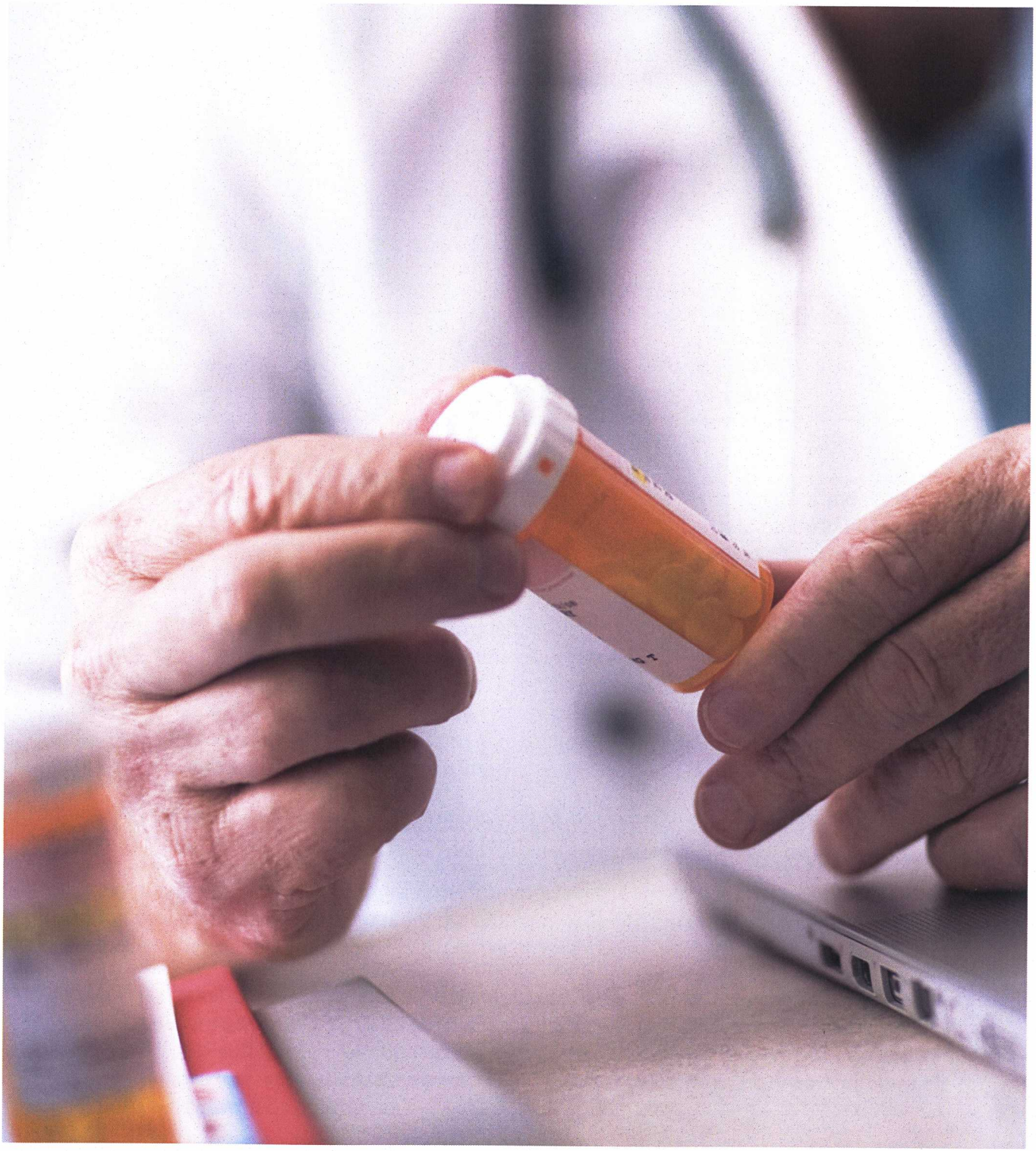
MoH Singapore has been regularly reviewing and adding more drugs to the MAF drug list since its launch and has increased the size of the fund accordingly[82]. Both the MAF and the Samaritan Fund apply a targeted approach to improve the access of unlisted high cost but clinically necessary medicines to eligible patients.

A similar arrangement by the Malaysia government known as "Tabung Bantuan Perubatan" (Medical Assistance Fund) is available for local needy patients. Limited information is available in the public domain about this fund in Malaysia. Still, Together Against Cancer (TAC), a coalition of local NGO groups, has commented that the national response to cancer has been inadequate and urged the government to set aside RM 50 million for a national cancer drug fund[83].

SHARING RESPONSIBILITIES BETWEEN THE PUBLIC AND INDIVIDUALS

Much of the accelerated growth in healthcare costs has been associated with the moral hazard issue, and many economists have sought to dampen inflation by reducing insurance coverage and increasing the patient's burden through copayments and deductibles[84]. Co-payment is a key feature of healthcare financing models in many countries including those in the region such as China, Hong Kong, Taiwan and South Korea. Meanwhile, deductibles, in addition to co-payments, are incorporated into the system before government subsidies are applied to qualified patients receiving care in public hospitals and polyclinics in countries such as Singapore. Outside of Asia, German Statutory Health Insurance (SHIs), for its part, reimburses to reference-price, with patients covering the difference out-of-pocket. Similarly, in countries such as the US, Sweden and Australia, patients who refuse generic substitution or choose a more expensive brand also pay the price difference.

Without patient access schemes, the proportion of 'not recommended' drugs in the UK would have been much higher[85]. Nevertheless, bureaucratic outcome-based schemes, which are hard to administer, are not welcomed by the payers. The complexities of the different risk-share schemes in operation can lead to providers being charged and reimbursed incorrectly, and errors in retrospective cost reconciliation. Addressing such situations requires significant extra work from pharmacy and finance departments, and



this administrative burden: negotiation, monitoring and evaluation costs can be substantial and therefore must be taken into account alongside savings in drug acquisition costs[86, 87]. A report from the British Oncology Pharmacy Association (BOPA) concluded that hospital pharmacies would rather not have to deal with patient access scheme (PAS) and would prefer a straightforward discount[88]. Good governance processes are also essential[89]. Manufacturers can nevertheless use the agreements to first get covered within a market, and then to build trust and good faith in their products with payers by proving their therapeutic value via real-world evidence collection[90]. At the same time, patients have the access to innovative medicines without unnecessary delays.

Chapter 5: Conclusions

Today, Malaysians generally enjoy free or highly subsidized care at public facilities. However, the rapid rise of incomes and the advance of medical technology have raised popular expectations more rapidly than the public system has been able to respond[91]. Being aware of these challenges, the Malaysian government has set into motion a number of initiatives designed to improve the access to and quality of healthcare to patients.

Our review and analysis of the success and challenge of achieving high quality and sustainable universal coverage while efficiently using limited healthcare resources, has led us to the following conclusions:

- Over the last decade, HTA has been implemented in a number of Asian countries. However, a lack of trained human resources, the limited availability and accessibility of reliable local resources and cost data and the inability to turn research output into policy and practice are key obstacles in conducting high quality local economic evaluations[22].
- An improved IT infrastructure—including electronic medical records, outcomes data and financial systems linked to a provider's database— would not only provide reliable local data but also be required to effectively manage the administration of alternative access schemes such as performance based schemes or coverage with evidence development.
- Every access model and scheme has pros and cons reflecting the point of view of the primary stakeholders—payers, clinicians, patients and manufacturers. Ultimately, the common goal is to achieve sustainable and equitable access of healthcare. To accomplish this, clarity, fairness and transparency are required—not just in evaluation scoping, process and methodology, but in evidence requirements, criteria, guidelines, decision-making rules, detailed explanations for decisions and listings of committee members and other stakeholders. A flexible approach that is open to negotiation is imperative, as is a timely management of diverse circumstances.
- High-quality communications among the various parties responsible for the evaluation and engagement process will be key to Malaysia as the future unfolds.
- Capitation may impede the neediest patients from getting the treatments they need. Lengthy listing evaluation processes should be avoided if the ultimate goal is to ensure that patients are treated with the medicines they need.
- While MoH is planning to improve the current formulary listing and financing mechanisms, access to innovative medicines at public facilities should not be delayed. In many countries such as the UK, Japan, South Korea and Taiwan, reimbursement application is accepted once marketing authorization is granted for a product. In fact in Australia, simultaneous reviews of regulatory and reimbursement applications are allowed by authorities.
- Alternative access schemes such as finance- or outcome-based agreements should be explored and implemented through a dedicated fund that will enable wider access to innovative therapies for critically ill patients in Malaysia.

In the absence of details in the proposed reforms to the National Medicines Policy in Malaysia, it is therefore both speculative and difficult to make in-depth recommendations regarding best next steps. However, in order to realize the vision of universal coverage, it is clear that Malaysia must seek out greater public funding and financing while flexibly exploring different possible access schemes in order to improve and provide sustainable access to innovative medicines.

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Innovating for a Healthier, Economically Vibrant Nation

OUR VISION

is to be an organisation working together with key stakeholders for better health and quality of life.

OUR MISSION

is to provide access to innovative medicines for better health and improved quality of life for all in Malaysia by:

* Promoting timely access to quality and innovative medicines
* Encouraging research and development of pharmaceutical products in Malaysia
* Forming strategic health partnership with key stakeholders for the advancement of public health
* Empowering consumers for safe and responsible self-medication
* Promoting industry values and contributing to the nation
* Upgrading the skills and knowledge of industry's human resources
* Ensuring the ethical promotion of medicines in compliance with local laws and a set of marketing practices

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