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Next Steps for Rare Diseases in Malaysia: Improving Access to treatments

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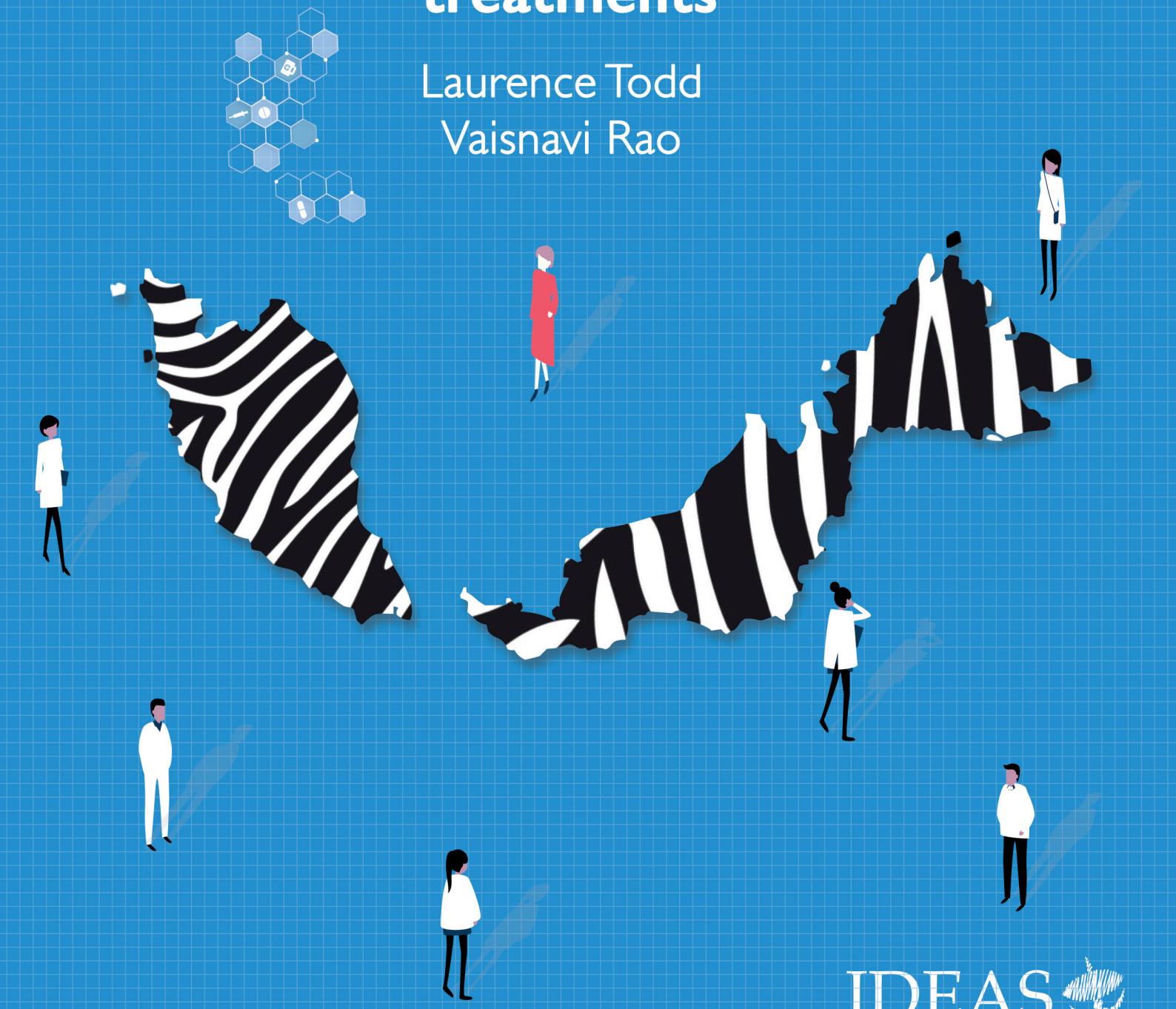

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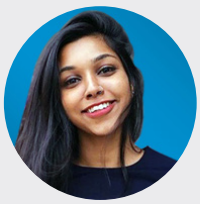

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Executive summary

The Malaysian government has committed to establishing a framework for managing rare diseases. With respect to improving access to treatments, the government has indicated it is interested to pursue the option of i) a trust fund and ii) increased regional collaboration. This Paper explores these two options to propose policy considerations for improving access to treatment for rare disease patients.

1.1 Trust fund

Malaysia's healthcare financing is fractured across a number of areas. There is universally available publicly funded healthcare, but the scope of coverage is limited. The government incentivises the use of private medical insurance, but the uptake remains low. Recently, the role of socialised insurance has increased through the Social Security Organisation (SOCSO/PERKESO) and the Employers Provident Fund (EPF), but the scope of these tools is limited.

The treatment of rare diseases poses a unique financing challenge. Treatment for rare diseases in the form of orphan drugs (ODs) are often expensive on a per-patient basis, given the small patient populations and the complexity of the treatments. As such, the funding of ODs is a challenge for healthcare systems around the world.

As a result, access to ODs in Malaysia is restricted. In Malaysia, high-cost ODs are provided on a case-by-case basis through a combination of private funds, pharmaceutical programmes, and government funding. However, available funding is insufficient to meet the demand for ODs.

The government has indicated that a trust fund be established to support access to ODs. In response to this challenge, the government has expressed an interest in establishing a trust fund that can leverage funds from the private sector and charitable donations to help to meet the cost of ODs.

Previous efforts to establish a trust fund and lessons from other countries highlight two key principles: sustainability and good governance. The paper considers other efforts to establish trust funds and what Malaysia can learn from these experiences: to be successful, the trust fund will need to be financially sustainable and well governed.

To ensure sustainability, the government will need to allocate the necessary public funds as part of a broader strategy to leverage financing. Fundraising by patient groups alone will not be sufficient to achieve financial sustainability. The government will need to consider how to allocate public finances to provide a foundation for a sustainable fund and to leverage charitable donations, particularly from the corporate sector.

To ensure good governance, the government will need to decide on objective criteria for access and prioritisation and the relation of the trust fund to the wider healthcare system. There will be a need for clear and transparent criteria to determine the eligibility of treatments available through the trust fund and patients' access to those treatments. Decision-making will need to be clear and transparent and reduce the scope for conflict of interest. Final decision will ultimately need to rest with the Ministry of Health.

1.2 Regional co-operation

Regional collaboration can support countries to widen access to treatment for rare diseases. Cross-border collaborations between European countries, with the objective of improving rare disease management and access to these high-cost treatments, offer useful insights into regional collaboration methods that could be adapted for Malaysia and other countries in the region.

Based on experiences from other countries, Malaysia should prioritise a flexible approach to regional collaboration. Any regional collaboration platforms should have flexible grouping with staged goals while taking into account the existing capacity and fiscal abilities of different countries. Established regional collaboration platforms may represent united goals but outcomes should remain flexible, with independent implementation in respective countries.

Malaysia should embark on efforts to improve regional collaboration, building on existing platforms, including APEC. As the host of Asia Pacific Economic Cooperation (APEC) 2020, it is an opportune time for Malaysia to develop conversations on improving collaboration for rare disease treatments at the regional level and move towards achieving the 2025 regional collaboration targets in the APEC Rare Disease Action Plan.
consistently in the MEB Annual Reports. The focus on certain indicators diverts the effort on other aspects that are equally important to address the challenges.

2. Access to orphan drugs in Malaysia

2.1 Malaysia's healthcare financing

Malaysia's healthcare system operates on the basis of mixed financing. Public healthcare is available to all citizens, financed by the Ministry of Health from general taxation. This system provides a basis for universal healthcare, but in practice the public healthcare system is insufficient to meet the healthcare demands of the population; public healthcare expenditure only accounts for about half of all healthcare expenditure in Malaysia[1]. Relative to the size of the economy, public expenditure on healthcare amounted to 2.35% of the GDP in 2019[2], an increase of 0.09% from 2018 following a manifesto commitment by the Pakatan Harapan government to increase healthcare spending to 4% of GDP.

The availability of treatments in the public healthcare system is usually decided according to the Health technology assessment (HTA) process, which determines whether a proposed health technology addresses a medical need and represents value for money. If so, it is made available for use in the public healthcare system. For drugs, this means being listed on the Formulari Ubat Kementerian Kesihatan Malaysia (Formulary). The decision of whether or not to prescribe a specific treatment is also constrained by budgets at the hospital and ministry levels. Under certain circumstances, specific additional budget allocations can be provided by the Health Minister, including for treatments not listed on the formulary.[3]

1. *Thong et al., 2019*
2. *Zaidi et al., 2019*
3. *Shafie, 2019*

Non-public healthcare expenditure is primarily comprised of out-of-pocket expenditure (OOP), which accounted for 36% of healthcare expenditure in 2013. It is important to note that Malaysia has a successful medical tourism industry, which may partly explain the high percentage of private payments. However, it also underlines the modest scope of private and social insurance mechanisms.

Private insurance is available within the private sector, with variable premiums charged based on the individual's health status, the type of health insurance, and the level of coverage. In some cases, employers may choose to offer health insurance coverage for their employees as part of a wider benefits package. Despite government efforts to increase the uptake of private insurance through various tax incentives, expenditure from private insurance remains relatively low at 8% of the total healthcare expenditure.

Social insurance mechanisms have been enhanced in recent years, but coverage remains low. The Social Security Organization (SOCSO) provides coverage for work-related injuries and support for members taking medical leave based on a system of statutory contributions. The Employers Provident Fund (EPF) allows members to make withdrawals to cover healthcare expenses under certain circumstances, although this is less a social insurance and more a form of socialised savings. Despite these initiatives, social insurance accounts for only 1% of healthcare expenditure in Malaysia.

2.2 Current access to orphan drugs

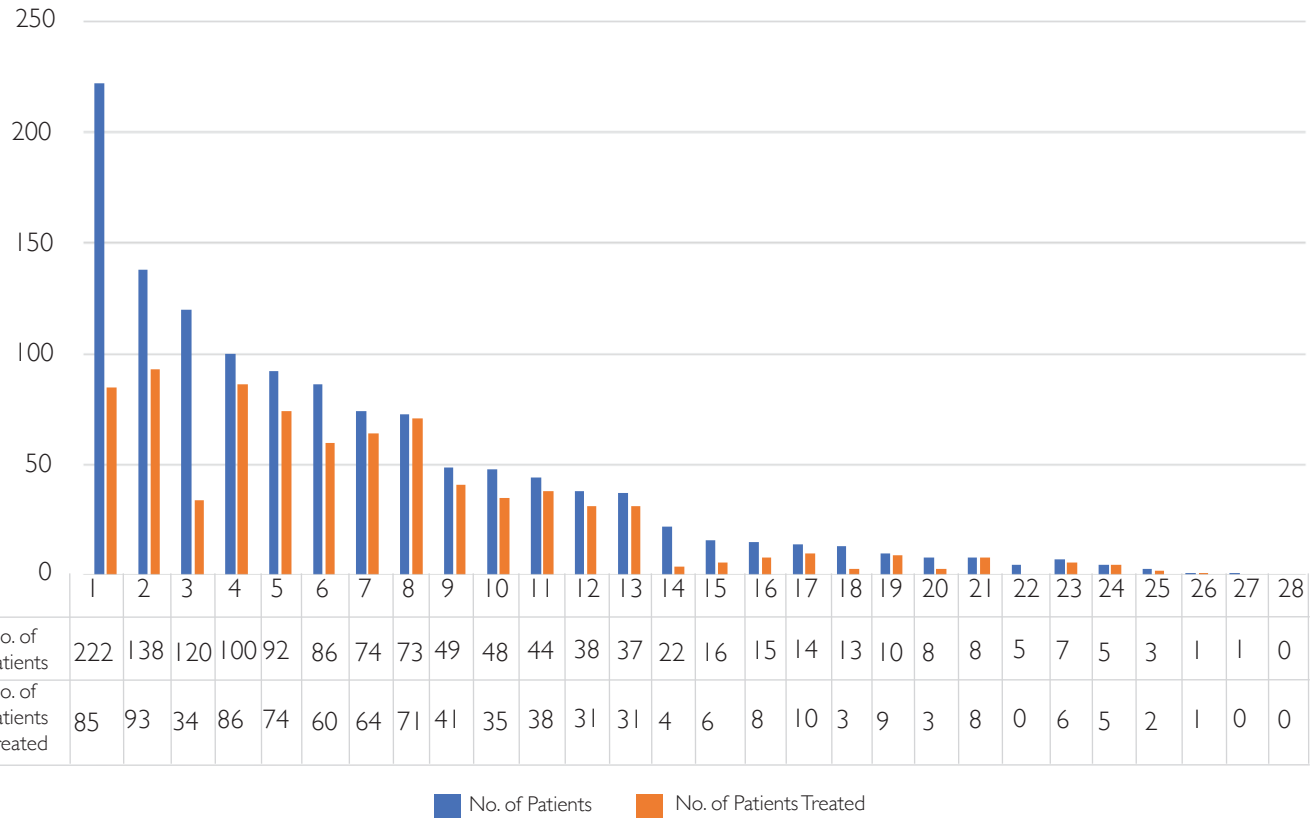
ODs represent a unique challenge for healthcare financing around the world. The per-patient cost of some treatments can be exceptionally high, often breaching the normal cost-effectiveness ratios used by public healthcare systems. However, the number of potential patients is, by the nature of the rarity of the diseases, very small; the total cost of making ODs available can be small relative to healthcare expenditure overall. In response to the particular challenge posed by ODs, many public healthcare systems have adapted their HTA processes to accommodate ODs[4]. This can include using a higher cost-effectiveness threshold, or the use of multi-criteria decision analysis (MCDA) to take into account a broader number of factors in determining value for money. The underlying tension that all healthcare systems face with respect to high-cost ODs is between equity – ensuring that resources are distributed fairly and consistently – or non-abandonment – ensuring that if a patient's life can be saved, then it is.

This tension is also present in Malaysia's policy on ODs. As outlined in Shafie (2019), the emphasis on affordability in the current conventional HTA method as applied in the public healthcare system leaves rare disease treatments at a disadvantage, given the high unit cost of rare disease treatment despite the low number of patients; the total expenditure of orphan drugs would be similar or lower overall than other diseases. Amending the HTA to use other methods such as MCDA that allow clear trade-offs between other criteria – such as rarity and unavailability of alternative treatments – would increase the number orphan drugs that can be listed in the formulary. However, this would not solve the problem of access entirely as the budget allocated is limited, resulting in a gap in access to treatments approved ODs. As a result, access to ODs is provided on the public healthcare system only on a partial basis.

[4] *See Thong et al. 2019 (fiscal analysis section) for detailed discussions on this.*

The prevalence of rare diseases in Malaysia is unknown as a registry has not yet been officially established. However, recently available data and research show that 1,249 patients were diagnosed with rare diseases in Malaysia as of June 2015 (Shafie et al., 2020). Using a list of 28 groups of rare diseases based on World Health Organization (WHO) classification, a cross-sectional study was carried out to estimate access to treatment in Malaysia. Approximately 60% of these patients received treatment, while the remainder underwent symptomatic treatments as of June 2015 (see Figure 1).

Figure 1: Rare disease group: Number of patients and patients treated

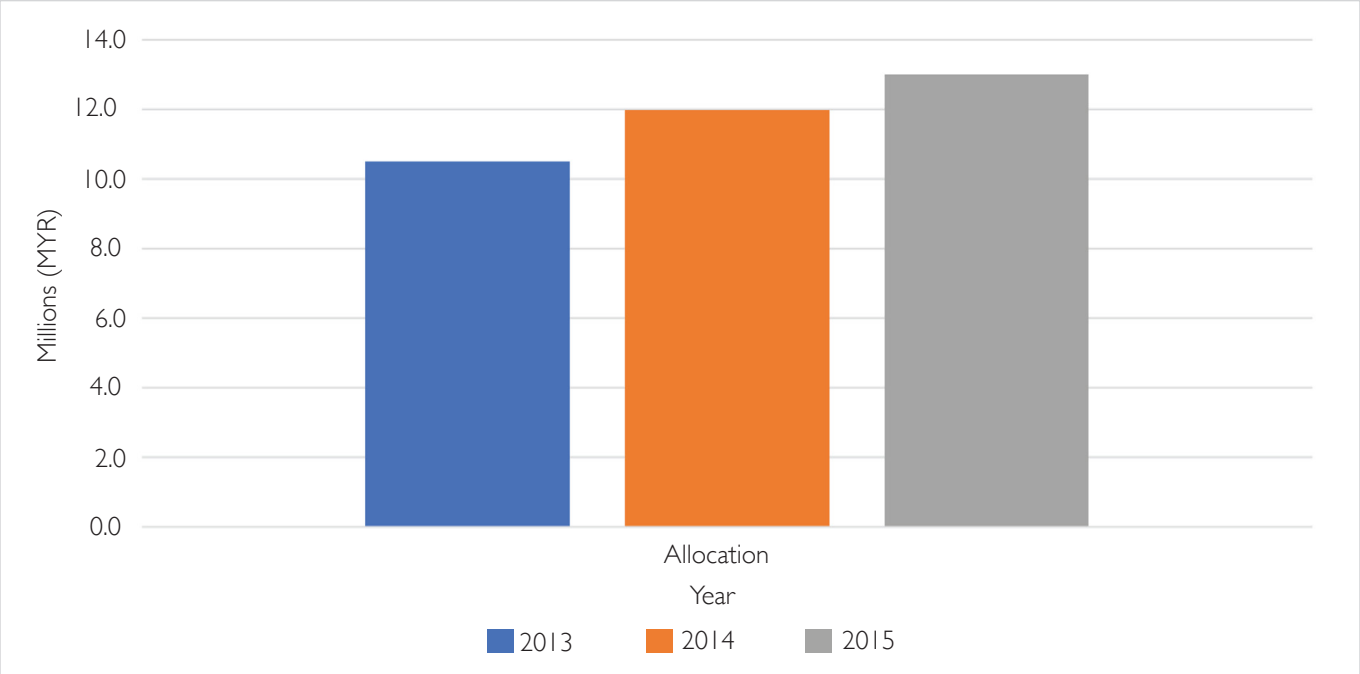


	1	2	3	4	5	6	7	8	9	10	11	12	13	14	15	16	17	18	19	20	21	22	23	24	25	26	27	28
No. of Patients	222	138	120	100	92	86	74	73	49	48	44	38	37	22	16	15	14	13	10	8	8	5	7	5	3	1	1	0
No. of Patients Treated	85	93	34	86	74	60	64	71	41	35	38	31	31	4	6	8	10	3	9	3	8	0	6	5	2	1	0	0

Source: Shafie et al. (2020)

To fund access to treatment, the government allocation for rare diseases has increased gradually over the last few years (see Figure 2); however, it is only secured for a rolling six-month period, lacking sustainability. This allocation is solely for medicine specific to rare disease treatment, excluding standard medicine and items (Shafie et al. 2020). Further expansion of this allocation is subject to fiscal constraints and the lack of an overarching policy direction on rare disease treatment.

Figure 2: Allocation for Genetic Clinic, Hospital Kuala Lumpur



Source: Shafie et al. (2020)

Private insurance policies in Malaysia do not cover ODs, partly because of the lack of a centralised registry of rare disease patients. Additionally, the use of ODs means that insurance companies lack the data to undertake the actuarial analysis to determine the viability of new rare-disease-inclusive products[5]. The current social insurance mechanisms are also insufficient to cover the cost of ODs. SOCSO benefits are tied to employees and their contributions; rare disease patients are often children and EPF withdrawals are simply too small to cover the cost of ODs, leaving members with less savings for retirement. Therefore, the current healthcare financing mechanisms in Malaysia are ill-suited to manage the costs of ODs.

Alternative funding mechanisms that do not rely on the consistent expansion of the fiscal space in the public healthcare system must be considered.

5. *Interview with the insurance industry.*

2.3 Malaysia rare disease framework

In 2019, the Ministry of Health formed a national framework and committee to integrate the management of rare diseases in Malaysia. The National Framework for Rare Disease has been approved by the ministry and is being implemented at time of writing. The framework includes the provision of a clear definition of rare disease, a review of the HTA process for ODs, and addresses the question of financing. The precise policy outcomes with respect to financing are not yet finalised.

Given the aforementioned challenges, the federal government has indicated that the favoured financing option for the expansion of access to ODs is the use of a trust fund[6]. While the precise parameters for how the trust fund would operate have not been decided, we understand that the premise is to leverage non-public funding sources – specifically donations – to finance wider access to treatment.

3. Rare diseases trust fund

3.1 International healthcare trust funds

A national trust fund is a pool of funds from various sources of funding, such as local taxes, charitable donations, and private-sector contributions, that are allocated for a specific purpose. The size of the fund is dependent on the purpose of the fund and the type of financing mechanism used. For example, in cases where the interest rather than the principle is used, a more reliable flow can be produced as the investments can generate a steady return while the initial principle investment is untouched. However, this requires a larger up-front investment.

Trust funds have been used in healthcare systems in other countries. The most relevant example to consider is Singapore. Its Rare Disease Fund is a charity fund that is supported through the combination of community donations and government matching contributions to support treatment. As of November 2019, the fund was expanded to support a total of six lifesaving medicines (see Table 1).

Table 1: Life-saving medicines covered under the RDF

Condition	Medicine
Primary bile acid synthesis disorder	Cholic acid
Gaucher disease (type 1 or 3)	Imiglucerase (Cerezyme) Velagluverase alfa (VPRIV) Taligluverase alfa (Elelyso)
Hyperphenylalaninaemia due to tetrahydrobiopterin (BH4) deficiency	Sapropterin dihydrochloride (Kuvan)
Pompe Disease (New)	Alglucosidase alfa (Myozyme) – new (2019)

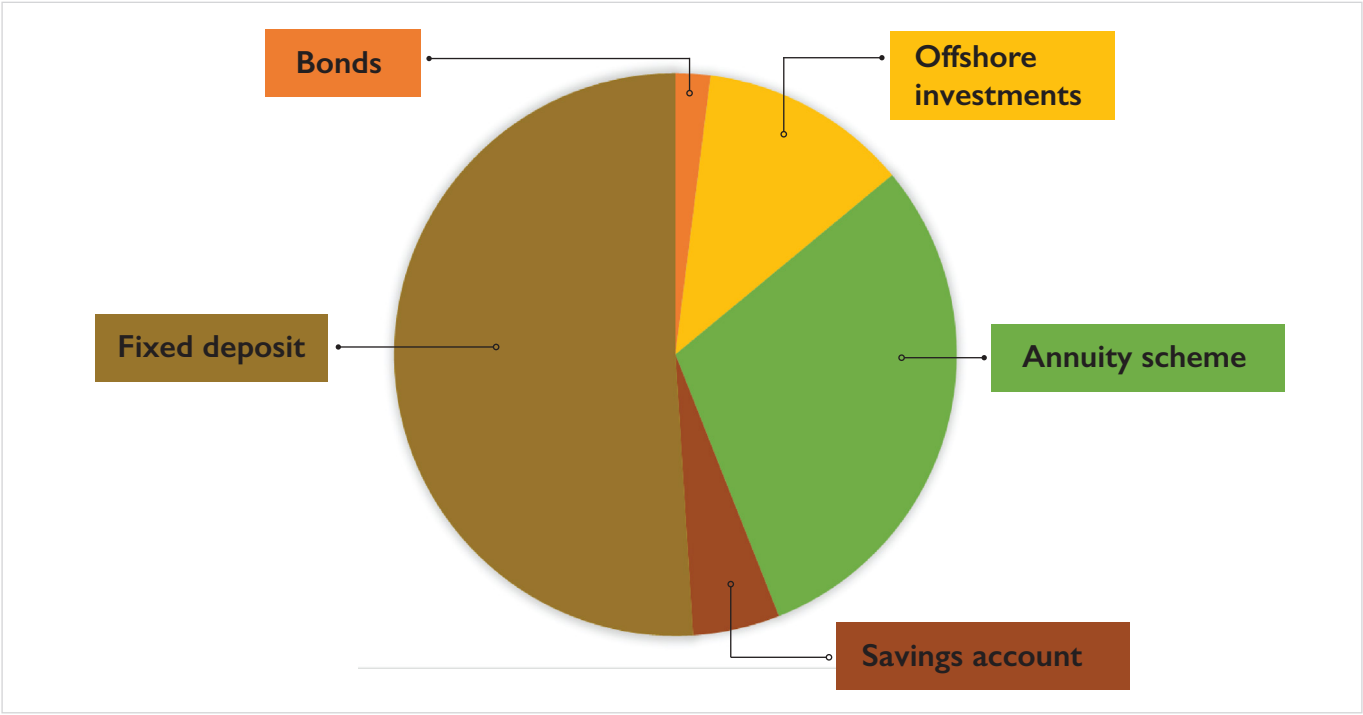
Source: RDF website

[6] *Interview with government officials.*

For comparison, we can also consider the healthcare wide trust fund developed in Bhutan. In 1998, the government of Bhutan launched the Bhutan Health Trust Fund to provide sustainable funding for healthcare items such as vaccines, essential drugs, and important equipment such as needles and syringes that are sensitive to price fluctuations in the healthcare system.

The government financed half of the needed USD24 million, with the remainder coming from a combination of different private and public donors. The trust fund was legally incorporated in April 2000 with an established secretariat and a governing board that includes members from the Ministry of Health, the Aid and Debt Management Department, and the Royal Monetary Authority of Bhutan. As of 2019, the trust fund consists of funds collected in the forms of accrued interest received from investments in fixed deposits, deposits in the savings account, bonds and annuity schemes in local financial institutions, investments in the off- shore market in fixed deposit, and health contribution collected from a local working group (see Figure 3).

Figure 3: Bhutan Health Trust Fund Sources of funds—2019



Source: Bhutan Health Trust Fund

Although not a trust fund per se, the Cancer Drugs Fund (CDF) in England also provides a useful point of comparison. The CDF is an annual ring-fenced budgetary allocation specifically used to fund access to cancer treatments which have not yet been approved for routine commissioning on the NHS.

Table 2: Comparison of health trust funds

Country	Rare Disease Trust Fund (Singapore)	Health Trust Fund (Bhutan)	Cancer Drugs Fund (England)
Fund size	S$90 million (as of November 2019)	Nu. 3,084,102,267.23	GBP340 million
Fund type	Endowment – interest generated is used to support patients	Endowment and investments – accrued interest used to finance procurement	Ring-fenced budget allocation
Financing source	Government and private donations (3 to 1 government matching of donations)	Government and donations (various investment strategies – see Figure 3)	Government budget
Scope	To provide access to rare disease treatment for patients who cannot afford access	To finance procurement of essential drugs and vaccines in Bhutan	To provide access to cancer treatments that show promise but are not yet approved for routine commissioning
Incentives	Donations are 250% tax deductible	Tax-free status	N/A

Source: RDF, Bhutan Health Trust Fund, NICE

Lessons from these international examples are incorporated into the discussion below.

3.2 Trust funds in the Malaysian healthcare system

Trust funds already operate in the Malaysian healthcare system. For example, the Chronic Diseases Trust Fund is available to support expenditure linked to certain requirements, such as wheelchairs. The financial details of the trust fund are not publicly available, but it is intended to meet "one-off" costs rather than provide funding for ongoing treatment[7]. Therefore, it is not directly applicable to the rare disease case, though it does provide a precedent for a trust fund in the healthcare system

For rare diseases, a foundation *(yayasan)* already exists. Following the 2nd Malaysia Conference on Rare Disorders 2013, Malaysian rare disease patient support groups Malaysian Rare Disorders Society (MRDS), Malaysia Lysosomal Diseases Association (MLDA), and Malaysia Metabolic Society (MMS) came together to form a national alliance[8]. The Rare Diseases Alliance Foundation Malaysia (RDAFM) was subsequently established as a trust registered under the Trustees (Incorporation) Act 1952 in September 2017.

The government has indicated its expectation that RDAFM provide the vehicle for the rare disease trust fund and is willing to provide the necessary support to enable this. However, there are a number of challenges for RDAFM to perform this function relating to the sustainability and governance of the trust fund.

7. *Interview with HKL*
8. *RDAFM Website*

3.3 Sustainability of rare disease trust fund

The first challenge is ensuring that the proposed trust fund is able to meet the financial demand for treatments on an ongoing basis. It is understood that the primary expectation from the government is that the trust fund will be sustained by fundraising efforts led by patient groups.

Patient groups, representing patients and their families, currently engage in various fundraising activities, including charity events. Funds raised are allocated to beneficiaries according to the processes of the respective patient groups to cover the costs of treatment and provide additional income support to affected families[9].

Despite impressive fundraising efforts by patient groups, the funds raised have been insufficient to meet the costs of expensive treatments on a sustainable basis. Funds are often directed at income support or to support the initial costs of treatment. For example, funds raised for lysosomal storage disorders (LSD) are only able to cover the initial period of enzyme replacement therapy (ERT) for three months[10].

Charitable fundraising is made more difficult by a lack of interest from the corporate sector. The majority of funds raised from patient groups are based on personal relationships or networks. However, large donations would require the engagement on the corporate sector. This is complicated in Malaysia by a number of factors. Firstly, awareness of rare diseases as an issue is low. Secondly, the high cost of ODs often means that the number of beneficiaries that can be supported by a donation do not meet the criteria for corporate social responsibility (CSR) programmes.

It is also important to recognise that patient groups representing rare disease patients are not a single homogenous group representing one disease. There are many different types of rare diseases, each with different needs and priorities. While patient groups do co-operate to raise awareness of the overall challenges of living with rare diseases, this does not mean that their fundraising interests are fully aligned. Put simply: why would patient groups pool their fundraising efforts instead of focusing on their causes?

It is clear that patient-group-led fundraising will not be sufficient to meet the financial demands of a sustainable trust fund. Based on the experience of the specialist funds highlighted in Table 2, we can identify various other mechanisms to ensure the sustainability of the fund:

Ring-fenced annual allocation. The CDF in the United Kingdom is financed through an annual ring-fenced allocation. In practice, this already exists in Malaysia for rare diseases (see Figure 1) and could be absorbed into the trust fund. Without further measures to increase the available funds, this would in practice be a continuation of the status quo.

Endowment. In place of an annual budgetary allocation, the government could provide an initial endowment sufficient to generate interest to finance treatments rather than relying on an annual allocation. In Singapore, the government provided an initial allocation for this reason and future public finance contribution would be in the form of matching funds and providing incentives for private donations. In Malaysia, this would require a significant up-front investment, given the larger population.

[9] *Interview with patient group*
[10] *Interview with MLDA*

Tax incentives. The government can also play a role in catalysing private donations through the use of incentives. In Singapore, donations to the RDF enjoy a 250% tax deduction since the RDF enjoys the "Institutions of a Public Character" status. In Malaysia, taxpayers may file claims for deductible treatment of donations pursuant to Section 34(6)(h) of the Income Tax Act, 1967. For corporations, a deduction is allowed for cash donations to approved institutions. The deduction is limited to 10% of the aggregate income of that company for a year of assessment[11]. There have been specific examples of the government introducing more generous incentives (e.g. double deductions) on a limited-time basis for specific causes. On the assumption that the trust fund would be an approved institution, donors would – at a minimum – benefit from the standard tax deduction[12]. Introducing a more generous tax incentive (i.e. double deduction) would likely incentivise larger donations, although it raises the question of what level of public financial support should be offered to rare diseases compared with other charitable causes.

Match-funding. An alternative to tax incentives is match-funding. The Singapore RDF operates on the basis of a 3-to-1 match-funding, where the government contributes SGD3 for every SGD1 privately donated. The Malaysian government has supported patient group fundraising in the past, but on an ad hoc basis. In 2014, the government supported fundraising efforts by the MLDA with a RM3 million contribution as part of their fundraising efforts. However, this was on a one-off basis and is not part of a broader strategy to match or otherwise directly support fundraising efforts by patient groups. Match-funding can be an alternative or complement to tax incentives to use public finances to leverage charitable donations.

Raising awareness and building confidence. Beyond direct financial support, the government can also play an important role in raising awareness and support among private donors. Awareness of rare diseases is low, which reduces the scope of fundraising. Moreover, sustainable funding will require large donations that are more likely to come from the corporate sector[13] – but awareness of rare diseases is also low in the corporate sector and the high cost of treatment can fall foul of corporate beneficiary requirements. The government can play an important role in convincing private donors that supporting rare disease treatments is an important and legitimate cause.

To achieve sustainability, the proposed rare disease trust fund will need to utilise at least some of the mechanisms above.

3.4 Governance of the rare disease trust fund

The second challenge is how the trust fund is governed. Eligibility would need to be determined in two respects: what is considered to be an eligible treatment or other expenditure, and which patients would be eligible for access.

In the case of the CDF in England, this is effectively part of the commissioning process managed by the National Institute for Clinical Excellence (NICE). The CDF provides NICE with a new recommendation that can be given for cancer treatments. The recommendation – "recommended for use within the CDF" – will be used where there is plausible potential for a drug to satisfy the criteria for routine commissioning but too much uncertainty surrounding the clinical data and cost effectiveness to make such a recommendation[14]. In

[11] *See Section 44(6) of the Income Tax Act.*
[12] *Not all patient groups have or have been able to maintain tax-exempt status.*
[13] *According to the RDF website, the largest donations for RDF in Singapore are corporate donations.*
[14] *Appraisal and Funding of Cancer Drugs from July 2016 (including the new Cancer Drugs Fund), NICE 2016.*

other words, the high cost itself is not a factor per se, but insufficient evidence to determine whether a given cancer drug meets NICE routine commissioning criteria, including value for money. The previous version of the CDF (prior to 2016) was criticised for effectively applying a higher cost-effectiveness threshold than is used for wider NHS commissioning[15]. While the CDF has no relation to rare diseases, NHS England employs a separate set of cost-effectiveness criteria for rare disease treatments, although not as part of a separate ring-fenced fund[16]. Since NHS England is a universal healthcare system that is free at the point of access, all NHS patients are eligible for the CDF.

In Singapore, the RDF is subject to a unique set of governance mechanisms that sit alongside the wider system for public healthcare commissioning:

- Prospective RDF treatments are prioritised for evaluation by the Agency for Care Effectiveness (ACE) in consultation with the Ministry of Health's Rare Disease Expert Working Group (RDEG). The RDEG consists of local clinical experts with experience in the treatment of rare diseases[17].

- The ACE technical team prepares a clinical briefing document for each treatment selected for evaluation in consultation with RDEG, which includes a summary of published clinical evidence, funding decisions from overseas reference agencies, local costing information and published prices in five overseas reference countries/regions (Australia, New Zealand, the United Kingdom, South Korea, and Taiwan) where available.

- The RDF is overseen by a voluntary RDF Committee comprising community representatives who approve the medicines covered under the RDF and determine the amount of financial support for each eligible patient according to their needs. The Women's and Children's Hospital (KKH) has been appointed as the Secretariat of the RDF Committee. Recommendations from RDEG and ACE's clinical briefing document are shared with the RDF Committee to inform their deliberations about which medicines should be included in the RDF. The recommendations are not binding.

All treatments made available on the RDF through this process must meet all of the following criteria[18]:

- Medicine is registered by the Health Sciences Authority (HSA) or a reputed international regulatory authority (Food and Drug Administration (US FDA) and/or European Medicines

15. Leigh and Granby (2016) found that the CDF exhibits a willingness-to-pay value of £223,627 per QALY, with 74% and 33% of expenditure for drugs with incremental cost-effectiveness ratios of more than £50,000 and more than £90,000, respectively. During 2013–14, CDF expenditure generated 4,677 QALYs, compared with a potential 13,485 if the same funds were used as part of routine NHS commissioning, displacing 8,808 QALYs. By ring fencing 10%, 25%, and 50% of the CDF budget for the provision of unevaluated drugs, cost-effectiveness thresholds of £149,000, £111,400, and £68,600 were calculated, respectively.

16. Following changes introduced in April 2017, NICE set a maximum additional QALY threshold of £300,000 for highly specialised treatments, under which they will automatically be approved for routine commissioning. This is 10 times higher than the standard NICE threshold of £30,000 for non-specialised treatments. The upper limit will vary according to the lifelong impact of the technology on the patient, ranging from £100,000 per quality-adjusted life year for treatments that deliver less than 10 QALYs to the patient in their lifetime, up to a maximum of £300,000 for treatments that deliver more than 30 additional QALYs to the patient in their lifetime.

17. The role of RDEG is to provide information regarding the estimated number of patients with specific rare diseases in Singapore and current clinical practice for the management of their conditions; provide advice on medicines that meet the eligibility criteria for inclusion in the RDF; address any clinical questions about specific rare diseases or treatments; and propose initiation and continuation clinical criteria for each treatment listed on the RDF to ensure treatments are used appropriately and that only patients who have an adequate clinical response to treatment continue to receive funding.

18. Drug Evaluation Methods and Process Guide, Agency for Care Effectiveness.

Agency (EMA)) for the condition assessed (i.e. medicine has proven therapeutic modality).

- Medicine treats a rare but clinically defined genetic condition that is chronically debilitating or life-threatening; there is acceptable evidence that the condition causes a significant reduction in either absolute or relative age-specific life expectancy or quality of life for patients with the condition.
- There is acceptable evidence that the medicine is likely to substantially extend a patient's lifespan and improve their quality of life as a direct consequence of its use.
- There is no cheaper alternative option (including non-drug therapy) for the condition.
- The medicine is not indicated for the treatment of other conditions, or if it is, the cumulative prevalence across all indications still falls within the definition of rare.
- The annual cost of the medicine would constitute an unreasonable financial burden on the patient and/or their family or carer.

Although the RDF raises money through charitable donations, decisions on disbursement are still subject to the commissioning process, given that public money is also being used. In line with the wider healthcare system in Singapore, the RDF does not utilise a specific incremental cost-effectiveness ratio (ICER) threshold for medicine. The Drug Advisory Committee (which advises on the subsidising of medicines) does not use a precise maximum acceptable ICER because ICERs are not precise values and are associated with a degree of uncertainty. In the case of rare diseases and the RDF, the RDEG works with the ACE technical team to prepare an appraisal of a given medicine, including costing information and published prices in five overseas reference countries/regions (Australia, New Zealand, the United Kingdom, South Korea, and Taiwan)[19]. Funding support through the RDF will generally only be extended to a medicine if its price in Singapore is comparable and not higher than published prices in overseas reference countries. This ensures the prudent use of charity funds and the sustainability of the RDF.

Finally, access for patients is means tested. This aligns with the wider healthcare system in Singapore, whereby the extent to which patient access to healthcare is subsidised based on their ability to pay.

Implications for Malaysia

There are several lessons which can be drawn for Malaysia from the management of these funds.

The scope of the fund. It will be important to establish clearly the scope of the fund and whether it is intended to meet the wider costs of rare disease management or only the cost of certain high-cost ODs. The decision so far in this paper has centred on the financing of ODs. However, there are significant unmet needs with respect to other areas of the rare disease landscape in Malaysia. For example, Shafie et al. (2020) report that many rare disease tests have to be sent abroad to Australia, Japan, and Taiwan for analysis, adding to the total cost of treatment. Based on the examples of the CDF in England and the RDF in Singapore, the trust fund should be narrowly focused and be viewed as only part of the wider improvement for rare disease management in Malaysia.

[19] *Addendum 1: Evaluation methods and processes for drugs under consideration for inclusion in the Rare Disease Fund (RDF), Drug Evaluation Methods and Process Guide, Agency for Care Effectiveness.*

Eligibility of treatment. The criteria for which treatments are eligible for the trust fund will need to be clearly defined. For this, the criteria for the Singapore RDF provide a useful starting point. In Malaysia, cost-effectiveness estimates are used to inform decisions on the procurement of drugs for use in the public health system, but there is no explicit cost-effectiveness threshold to decide on the reimbursement of drugs[20]; each decision is made on a case-by-case basis. Currently, there is no separate guidance on cost-effectiveness analysis for ODs. The establishment of the trust fund will require clarifying cost-effectiveness guidance for ODs, including the use of reference pricing, in relation to wider guidance on the procurement of drugs.

Eligibility of patients. The public healthcare system in Malaysia is available to all Malaysian citizens without any means testing. There is a case to consider whether some form of means testing or co-payment is appropriate for use of the trust fund. However, in most cases the cost of ODs is such that it is not possible for patients to directly bear anything but a small portion of the cost. Moreover, since there are currently no mechanisms for means testing in the public healthcare system it would be challenging to implement such a system strictly for access to ODs alone. Therefore, access would likely need to be provided on a universal basis.

Technical expertise. It is clear that decisions on the use of fund to meet the cost of treatment will require significant technical expertise. This includes clinical expertise to determine the need for a given treatment alongside other relevant technical expertise to prepare the necessary assessments to support decision-making, e.g. economic modelling. The trust fund would need the support of a committee of such experts.

Decision-making and conflict of interest. In the event that public finances are contributed to the trust fund (a likely scenario given the issues of sustainability), there will need to be public oversight of decision-making, likely though the Ministry of Health. Moreover, decision-making must not be undertaken by those who stand to benefit directly from the trust fund, i.e. the patients and their families, to reduce the scope for conflict of interest. Patient groups should play a consultative and advisory role and not have any direct influence over decision-making.

Suitability of the RDAFM. On the basis of these issues, the RDAFM in its current form is not equipped to govern the trust fund. Indeed, under the RDAFM trust deed, the foundation is not permitted to receive funds for ODs intended for the treatment of rare diseases[21]. Even if this constitutional barrier can be addressed, the RDAFM is not well-positioned to manage these other issues.

3.5 Trust Fund Recommendations

In light of the unique challenges posed by the funding of ODs, there is a case to establish an innovative financing mechanism in the form of a trust fund. However, to be successful the trust fund will need to be sustainable and well governed.

- Fundraising efforts by patient groups alone will not be sustainable and there will need to be public financial contributions and government support for charitable fundraising efforts.

- The trust fund will also need clear governance with well-defined eligibility criteria for treatments and patients, with the appropriate decision-making body supported by technical experts.

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[20] *The 2019 Pharmacoeconomic Guidelines for Malaysia provide the current discussion on the use of an explicit Cost Effectiveness Threshold (CET)*

[21] *Section 3.3(b) of RDAFM constitution; interview with RDAFM*

For these reasons, the RDAFM in its current form is not suitable to meet test of sustainability or good governance. The table below offers the following recommendations:

Table 3: Trust fund recommendations

Topic	Recommendations
Public finance contribution	• Absorb ring-fenced allocation within the trust fund as the basis for an annual budget OR • Endow the fund with sufficient capital to generate interest
Financial incentives for charitable donations	• As a minimum, ensure donations to the trust fund are tax deductible AND/OR • Introduce match-funding of charitable contributions AND/OR • Introduce more generous tax incentives (e.g. double deductions)
Confidence building for charitable incentives	• Take steps to raise awareness and build confidence in corporate donations for rare diseases (e.g. letters of support)
Scope of fund	• Define clearly the scope of the fund – if only for orphan drugs – then ensure other needs are met in the wider healthcare system
Treatment eligibility	• Define clear eligibility criteria for treatments accessible through the fund, consistent with the wider public healthcare system but recognising the unique challenges posed by rare diseases
Patient eligibility	• Define clear eligibility criteria for patients accessing the scheme, which could include some form of means testing
Governance	• The MOH should oversee the governance of the trust fund and be the ultimate decision maker AND • A decision-making committee representing other stakeholders should be established to support the MOH AND • A committee of technical experts including clinicians should be established to support decision-making

Source: RDF, Bhutan Health Trust Fund, NICE

4. Regional co-operation for Malaysia

Regional co-operation between the healthcare systems of Asia Pacific countries or ASEAN can benefit patients. There are different areas of co-operation that can take place between countries to improve access to medicines for patients. However, this healthcare co-operation varies based on the different scope, process, structure, and purpose of the collaborations. The goals for different areas of cross-border collaboration for healthcare systems include:

- **Joint horizon scanning:** The aim is to highlight important pharmaceutical innovations before they reach the market and continually gather data and analyse research and literature. This will provide insight into expected costs, and enables timely decision making and (joint) price negotiations.

  Joint Health Technology assessment: Building collaboration on a supranational level, leading to joint health technology assessments to explore new mechanisms, i.e.; the mutual recognition of national assessments to reduce workload of national HTA – organisations.

- **Information sharing:** Exchange information on medicine policies. Sharing of information and collaboration between countries, over an extended period of time, will benefit policy initiatives on managing access to treatment

- **Joint price negotiations or procurement:** Collaboration on price transparency is intended to enable countries to seek more transparency on the cost build-up of pharmaceutical products and therefore improve medicine pricing and access for patients. Ultimately this can lead to joint procurement of medicines as a means for pooling purchasing power and improving access.

Regional collaboration is most advanced in Europe, as will be discussed below. However, the APEC action plan on rare diseases also recommends APEC economies to advance international and regional collaboration by facilitating the sharing of best practices related to rare diseases. As Malaysia chairs the 2020 APEC forum, it is an opportune time to develop national strategies towards regional co-operation to improve access to rare disease treatments. In the following section, we will review existing regional co-operation efforts between different healthcare systems to understand the challenges and benefits associated with establishing a similar platform for Malaysia to improve patient access to high-cost rare disease treatments.

4.1 Beneluxa Initiative

The Beneluxa Initiative is a cross-border collaboration to ensure sustainable access to innovative medicines at affordable costs for patients. The Beneluxa Initiative began in April 2015, originally as a collaboration between the health ministers of Belgium and the Netherlands to carry out price negotiations specifically for orphan medicinal products. They were joined by Luxembourg in September 2015, Austria in June 2016, and Ireland in June 2018.

Countries involved in the Beneluxa Initiative aim to collaborate on joint horizon scanning, health technology assessments, exchange of information, and joint price negotiations. Joint health technology assessments were explored for four different drugs between 2015 and 2017. Horizon scanning is a key activity under Beneluxa that allows countries to be aware of pharmaceutical innovations before they reach the market. The main

activities of horizon scanning include the identification of new and emerging pharmaceutical products entering the market, filtering identified products based on scope and time horizon, prioritising filtered products for early assessment, and early assessment of these products based on available data. Broad collaboration on horizon scanning specifically on the identification and filtration through an external database was proposed[22] .This led to the development of the International Horizon Scanning Initiative (IHSI) by Beneluxa, with the aim of involving as many countries as possible. As the benefits of horizon scanning can benefit many countries, full participation in the Beneluxa Initiative is not required to join the IHSI. The flexibility of this approach highlights the needs-driven and adaptive approach of the Beneluxa Initiative[23].

Information is an important factor in prioritising and determining access to treatments. Therefore, the horizon scanning aspect of Beneluxa is valuable in gaining foresight on products currently in the pipeline and the competitive landscape in relation to unmet clinical needs to guide investment decisions. The aim of the IHSI is to create an open source, publicly available global database on pharmaceutical financial data, investors' reports, clinical studies, scientific literature, and more[24].

The IHSI database serves as a platform for information on companies' pipelines to help countries estimate budget impacts, as well as set priorities and begin negotiations earlier. However, the benefits of this database are dependent on how participating countries choose to make use of the wealth of centralized information available to them. Nevertheless, other than governments, healthcare professionals and public payers will be able to improve their planning based on the information available[25].

Through the Beneluxa Initiative, countries can share their experiences on pharmaceutical policy practice to explore possible best practices. This includes facilitating discussion on biosimilar uptake, which has resulted in a mapping exercise of the biosimilar landscape in the respective member countries. These countries have explored collaboration of patient registries; however, this has a been limited by technical and political challenges associated with joint registries.

Participating countries in the Beneluxa Initiative have resources invested for health technology assessments on a national level. Building on this existing expertise, Beneluxa wanted to develop supranational collaborations on HTA. In 2018, Belgium re used Netherland's existing report on SPINRAZA® (nusinersen) in collaboration with the pharmaceutical company Biogen Inc. This led to a joint negotiation on SPINRAZA by both countries. Reuse of parts of assessments were also carried out for other products; however, as of November 2019 "mutual recognition of work" is the only element of collaboration between countries. Therefore, joint HTA currently allows collaborating countries to benefit from each other's expertise, but they have yet to progress to full adoption of HTA reports which would reduce the workload of national HTA organizations, owing to legal constraints[26.]

The negotiation of SPINRAZA involved in all stages of collaboration, leading to a confidential deal between Belgium, the Netherlands, and the company. This gave patients in the respective countries access to the drug under different reimbursement conditions. In the Netherlands, SPINRAZA would be reimbursed for patients

22. *KCE Belgian Health Care Knowledge Centre, 2017*
23. *European Public Health Alliance, 2019*
24. *Open Market Consultation: Building a Horizon Scanning System (HSS), 12 November 2018.*
25. *European Public Health Alliance, 2020*
26. *"Beneluxa", n.d.*

up to the age of 9.5 years, subject to evidence on its effectiveness in older patients. In Belgium, all age groups would be covered – but the final price was not made public[27]. This highlights a key strength of this initiative: countries involved do not always need to move together to improve access[28].

4.2 Valletta Declaration Group

The Valletta Declaration Group (VDG) was founded in May 2017 in La Valetta by Cyprus, Greece, Italy, Malta, Portugal, and Spain and has since expanded to include Croatia, Ireland, Romania, and Slovenia. Like the Beneluxa Initiative, the VDG aims to improve patient access to new treatments and therapies while ensuring the sustainability of their health systems. Its focus and activities include[29]:

- Joint clinical assessments & health economic evaluation
- Sharing of information on specific medicines or categories of medicines with significant therapeutic value and high financial impact in view of:
    - Input in the negotiation phase
    - Renegotiation of contractual arrangements
- Information exchange on good practices regarding pricing and reimbursement of biosimilars
- Joint price negotiations for medicine
    - Choice of medicine
        - without or at the early stages of market authorization
        - innovative medicines including orphans
    - Criteria for prioritization of products
        - Interest of the therapeutic indications
        - Unmet clinical need
        - Cost of therapies
        - Expected volume of use and prevalence of the disease
        - Alternative treatments already approved for these indications
        - Level of cooperation with the marketing authorization holder
- Horizon scanning of innovative therapies
    - Sharing of pharmaco-therapeutical/effectiveness assessments of medicines.

[27] *Beneluxa Secretariat. About BeNeLuxA – press releases. Positive outcome of joint reimbursement negotiations on Spinraza, 12 July 2018 https://beneluxa.org/archive (accessed, 24 August 2020).*
[28] *European Public Health Alliance, 2019*
[29] *Coggi, Paolo (2018) The Valetta Declaration Coggi, Paolo (2018) The Valetta Declaration [PowerPoint presentation]. Available at https://rb.gy/hi8gap (accessed, 24 August 2020)*

Currently, the collaboration between 10 countries with over 160 million citizens represents 31.5% of Europe's population. The VDG's approach is towards creating one aggregated joint market through pooled procurement for large volumes. The group also focuses on oncology drugs, orphan drugs, and treatments for autoimmune diseases[30].

Despite similarities with the Beneluxa Initiative, the Valletta Group has been considered more disruptive in their approach towards advocating for improved access through price negotiation and regional collaboration. They are the first group to follow through to implement the aims of the World Health Assembly that called for greater price transparency in the medicines market.

It is noteworthy that this resolution was sponsored by some members of the VDG during the 72nd World Health Assembly. This draft resolution was proposed by European and non-European countries including Italy, Brazil, India, Malaysia, South Africa and Uganda. The resolution urged member states to share data on clinical results and prices.[31] International reception of the declaration was mixed. There was positive responses and support for the resolution from most country delegates including Switzerland, Spain & the African bloc of member states. However, Germany, the United Kingdom and Hungary disassociated itself from the resolution, on the grounds that the complexity of access was not taken into account, and Germany called for "sufficient assessment of potential implications for the involved health care systems"[32]. The scope of final resolution was amended to mitigate these concerns, including agreeing on voluntary action for transparency. Italy and the 10 other members of the VDG have implemented practical steps to pursue greater transparency. According to Malta's Deputy Prime Minister and Health Minister, Christopher Fearne, the group would likely start with an agreement to share their respective national prices that they pay for medicines and other health products, in confidence.

The Valetta Group continues to push for price transparency and has considered exploring collaboration with Beneluxa on horizon scanning, with the ambitious goal of multi-country price negotiation and procurement.[33,34]

4.3 Regional Collaboration: Lessons Learned

The two regional cooperation platforms have utilized different approaches that offer various benefits to participating countries and useful insights for any future regional cooperation initiatives. Whilst the VDG's approach on collaborations have been more political, Beneluxa has prioritized technical collaborations, both with the aim of joint negotiations and improved access to treatments.

Although the VDG's activities are similar to Beneluxa, the focus on joint negotiations by the former is a more drastic approach towards improving access to medicine through regional co-operation. The VDG appears to be moving in the direction of collaboration to improve price transparency and produce a more competitive and innovative market, by encouraging exchange of policy solutions to key issues relating to the access of medicines[35,36]

[30] *Coggi 2019; Grubert, 2019*
[31] *Fletcher, 2019; WHA resolution*
[32] *Fletcher, 2019*
[33] *Grubert (2019)*
[34] *Tabone, (2019)*
[35] *Coggi (2018)*
[36] *European Public Health Alliance (2019)*

The group aims to conduct joint assessments, envisioning that joint outcomes would be implemented in line with national legal frameworks. However, the industry has raised concerns over the uncertainties of national-level implementations of joint outcomes[37]. As noted by Yannis Natsis of the European Public Health Alliance (EPHA), while the VDG has gained political endorsement, there is a need to de-politicize the current process to allow technical policy staff to continue learning about the best way to collaborate with member states.

On the other hand, Beneluxa's successful negotiation of SPINRAZA suggests that negotiations that involve all stages of collaborations can be a more efficient way forward to ensure success. Currently, the VDG operates in the view that larger volumes would result in better prices; however, the goal of moving all 10 countries in unison has not produced positive results. Different countries have different procedures, timing of assessment and negotiation at the national level. Therefore, there is the challenge of harmonising joint assessment in a rapid manner to not create delays in the access to medicines. There is also a need to guarantee confidentiality throughout the process and in final agreed prices.[38]

On the national level, when working with pharmaceuticals, keeping up with the increasing and complex workload is a resource-intensive task. There is often only a small capacity of civil servants to rely on, especially with staff turnover issues. One benefit of the culture of collaboration is the ability to learn from civil servants in different countries and, most importantly, develop enough trust to exchange information. This can be a taxing task considering the linguistic and legal barriers, along with the diversity of healthcare systems between member countries. However, this co-operative nature is the building block to any regional collaboration for healthcare[39]. Hence, closer collaboration between subgroups may be a more practical way forward, with flexibility of national level implementations.

Nevertheless, the VDG's bigger picture approach of establishing strategic collaboration on pricing and reimbursement of medicines amongst national authorities and encouraging sharing of information, can be beneficial to improving access to treatment for participating countries in the long term.

4.4 Malaysian context

As a member and host of APEC, it is opportune for Malaysia to move towards APEC's 2025 target of achieving regional collaboration on rare diseases. The APEC Rare Disease Action Plan encourages APEC economies to improve regional collaboration in the following areas[40]:

- Consider mutual reliance of regulatory decisions from other APEC economies to improve harmonization across the region, as well as establish a regional network or partner to facilitate the sharing of best practices related to the policy, regulatory, and reimbursement decisions of rare diseases.

- Lead efforts to advance international and regional collaboration for research and development.

- Encourage regional networks for human resource capacity-building in medical and non-medical sectors, especially around specific rare diseases or clusters. Build on existing programs and centers

[37] *Coggi, 2018*
[38] *Coggi (2018)*
[39] *European Public Health Alliance (2019*
[40] *APEC Action Plan on Rare Diseases.*

to offer cross-border clinical training and internships. Encourage and provide opportunities for public-private partnerships in medical and non-medical training and investment in regional comprehensive clinics and regional centers of expertise.

- Leverage coordinated efforts and regional partnerships in balance with local data privacy policies to generate and capture sufficient quantity and quality of Asian genetic reference sequences and make them widely accessible and available to researchers and clinicians.

- Encourage a regional network of newborn screening programs to crowdsource interpretation of test results, promote collaboration and innovation in programmes, and cultivate the training and development of genetic counsellors.

- Collaborate with industry, academia, and patient organizations to assemble a regional network of centres of excellence for resident healthcare professionals to exchange clinical guidelines and techniques, share best practices, and encourage innovation of rare disease diagnoses and treatments.

- Work together across the APEC economies to determine the feasibility and preliminary design of a single regional registry focused on rare diseases for all APEC economies to access and use.

- Work with private and public researchers and academia to facilitate regional and international pooling of trial data to solve challenges related to small patient cohorts in any jurisdiction.

Currently, Malaysia's involvement in cross-border collaboration for health include ASEAN. The large population, close geographical proximity, and increasing global opportunities in pharmaceutical trade provide an opportunity for cross-border collaboration between ASEAN countries to reduce pharmaceutical expenditure. Presently, the main form of collaboration is harmonizing drug regulations in member countries, which have been gradually developed through the ASEAN Pharmaceuticals Project in 1979, leading to the implementation of the ASEAN Common Technical Dossier. These efforts can be effective tools to enable pharmaceutical trade in the region but does little to improve access to medicine. However, there is an opportunity to leverage on these existing collaboration and efforts to eventually improve access to medicines. (Lee et al., 2019)

Malaysia has also been involved in the Price Information Exchange of Essential Medicines (PIEMEDS), a regional platform that promotes the sharing of medicine prices procured by the public sector, initiated by the WHO's Regional Office for the Western Pacific ("PIEMEDS", n.d.) However, due to the voluntary nature of the initiative, response was poor and the coverage of medicine type is limited. (Lee et al., 2019)

A quick scan of the Ministry of Health website shows that Malaysia currently has 44 horizon scanning initiatives, which include identifying the effectiveness of Rituximab biosimilars, a treatment for rare cancer. Malaysia's participation as co-sponsor to the resolution on price transparency at the 72nd World Health Assembly suggest that there is a willingness to move in the direction of negotiations. However, this has yet to translate into practical steps forward at the national level. In the case of rare diseases, this is understandable as Malaysia is in the early stages of implementing its first national framework for rare diseases.

However, Malaysia can initiate discussions on joint registries and information sharing at the Asia Pacific level to study the feasibility of such collaborations. This could also be done at the ASEAN level by leveraging existing collaborations to develop information-sharing platforms similar to the IHSI or PIEMEDS.

4.5 Regional Cooperation recommendations

The two case studies provide an overview of the challenges and benefits of pursuing cross-border collaboration to improve access to high-cost treatments. Regional co-operation efforts do not need to only focus on centralised pooled procurement. Information sharing, horizon scanning, and joint HTAs are valuable tools to bring about the shared benefits of better pricing mechanisms. These existing regional collaboration platforms also provide key insights in establishing similar platforms in the Asia Pacific or ASEAN regions. Firstly, there should be flexibility in the grouping of countries, and this does not need to be restricted to specific regions. The countries involved should have similar goals in improving access to high-cost treatments, along with a similar level of development. Secondly, established platforms should progress through staged goals (i.e. starting with shared registries before progressing to joint HTAs). This would allow countries to have the flexibility to collaborate on some, but not all stages, based on their respective priorities.

Finally, countries should be able to carry out joint negotiations with the autonomy of separate reimbursement conditions according to their respective needs and fiscal capacity. Independent management on the national level may be more effective to avoid complications associated with joint implementations. A more flexible approach to collaboration can allow countries to take part in specific activities based on their national priority and capacity.

5. Conclusion

In conclusion, if the Malaysian government chooses to establish a trust fund to improve access to treatments for rare disease patients, a sustainable financing mechanism will need to be established. However, this should not be reliant on independent fundraising efforts of patient groups alone. Additional government support and/or public financial contributions will be necessary for trust fund to be successful. The scope of healthcare needs, treatments and patients' that can be supported through the fund should also be well defined and consistent with the wider public healthcare system. The Ministry of Health should oversee the governance of this fund with the support of a committee representing relevant stakeholders in the rare diseases network in Malaysia.

Table 4: Trust fund recommendations

Topic	Recommendations
Public finance contribution	• Absorb ring-fenced allocation within the trust fund as the basis for an annual budget OR • Endow the fund with sufficient capital to generate interest
Financial incentives for charitable donations	• As a minimum, ensure donations to the trust fund are tax deductible AND/OR • Introduce match-funding of charitable contributions AND/OR • Introduce more generous tax incentives (e.g. double deductions)
Confidence building for charitable incentives	• Take steps to raise awareness and build confidence in corporate donations for rare diseases (e.g. letters of support)
Scope of fund	• Define clearly the scope of the fund – if only for orphan drugs – then ensure other needs are met in the wider healthcare system
Treatment eligibility	• Define clear eligibility criteria for treatments accessible through the fund, consistent with the wider public healthcare system but recognising the unique challenges posed by rare diseases
Patient eligibility	• Define clear eligibility criteria for patients accessing the scheme, which could include some form of means testing

Governance	• The MOH should oversee the governance of the trust fund and be the ultimate decision maker AND • A decision-making committee representing other stakeholders should be established to support the MOH AND • A committee of technical experts including clinicians should be established to support decision-making

In the medium term, the government should move towards developing regional collaborations with other APEC/ ASEAN countries through joint horizon scanning, joint health technology assessments, exchange of information on medicine policies and/or joint price negotiations. These efforts should be pursued with a level of flexibility, in relevant areas of collaboration, that would ensure timely access to medicines for rare disease patients in Malaysia and other participating countries.

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