Executive Summary

A rare disease is any disease that affects a small percentage of the population and are frequently debilitating to patients. Timely access to the right treatment is crucial for effective disease management and delayed access contributes to poorer treatment outcomes and more severe disease complications. This policy paper "Improving access to orphan drugs in Malaysia" explores the challenges associated with accessing orphan drugs in Malaysia and proposes ways in which we can improve patient access to these drugs.

We explore the current situation, which includes difficulty in receiving diagnosis and obtaining treatment in time due to the lack of expertise along with limited treatment options. Orphan drugs are harder to obtain than other conventional drugs due to lack of economic incentive for manufacturers to develop drugs which can be attributed to the small market. This leads to orphan drugs being marketed at very high prices due to an absence of economies of scale in its sale. Low prevalence of the disease within healthcare settings and population in general results in a lack of understanding among physicians. The low prevalence makes it difficult to improve knowledge on the safety and efficacy of treatments through proper studies.

The paper explores different solutions to address these problems through two broader themes: controlling the high price of drugs and increasing the budget for orphan drugs. The solutions proposed under these themes are briefly outlined below;

Controlling high prices of drugs

- Drug prices could be controlled through a more appropriate application of the Health Technology Assessment (HTA) for drug price negotiation to better reflect the unique characteristics of rare diseases. Taking into account disease severity and lack of suitable alternative treatment would yield more accurate national value assessments.

- A Management Entry Agreement could be implemented to ensure early access to drugs at a reasonable price.

- The government could also establish a regional network to boost purchasing power and achieve economies of scale to obtain lower drug prices. However, this option needs to be scrutinised more closely due to differing economic status among members of ASEAN.

- Besides that, compulsory or voluntary licensing on orphan drugs could be implemented to purchase these drugs at a cheaper price.

- In relation to advancing research for discovery of orphan drugs, Malaysia could explore pooled funding options to reduce risk associated with investment in the development of drugs.

Increasing the budget for orphan drugs

- ☑ Increase awareness surrounding rare diseases through the engagement of multiple stakeholders including politicians, policymakers, ministries, healthcare providers, families, the public and patient advocacy groups.
- ☑ Establish a co-payment system where patients within the public health system pay for part of their treatment.
- ☑ Form partnerships with private insurance companies and explore appropriate methods to reduce patient's financial burden. Specific policies could be introduced in the form of private insurance or social insurance schemes.
- ☑ Ring-fence allocations could be established for an existing fund or through the creation of a trust fund that pools resources from different organisations.
- ☑ A mortgage could be applied to orphan drugs through an agreement between healthcare payer and drug manufacturer. This would allow the cost to be spread over the period during which it would accrue the benefits of the reduced downstream costs from averted diseases.

In conclusion, there is a need to establish a separate fit-for-purpose framework to evaluate and fund rare diseases. The government should explore a new financial model to ensure rare disease patients receive the care they need as outlined in this paper. The government should also improve the access of treatment for patients of rare diseases, through various measures such as internal funding for rare diseases, which should be transparent and inclusive.