

## 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.