

# Executive Summary

## Solidarity Amidst Rarity: Reforms for Equitable and Sustainable Rare Disease Management

While the accessibility of healthcare in Malaysia has undoubtedly progressed considerably in recent years, the same cannot be said for patients grappling with rare diseases. Due to a combination of a lack of public awareness, a subpar standard of knowledge among professionals, and a dearth of diagnostic facilities, rare disease patients face significant delays in healthcare access. With the majority of healthcare facilities and expertise concentrated in the Klang Valley, location has become a prohibitive physical barrier for those residing outside the vicinity.





Furthermore, the high costs of diagnostic tests and treatment for rare diseases are not routinely funded by the government, necessitating short-term funding from external schemes, or requiring out-of-pocket funds which potentially leaves rare disease patients economically vulnerable. This, coupled with the lack of entitlement to the usual forms of social healthcare protection, result in rare disease patients being left to fend for themselves at the expense of their physical, financial, and emotional well-being.

In order to identify and measure healthcare access for rare diseases throughout the patient pathway, this study employed Levesque's framework to conceptualise healthcare access from the perspectives of the healthcare system and patients. Based on these barriers, this paper recommends four critical pillars to improve healthcare access for rare disease patients and stratifies them based on their complexities. The contents and the policy recommendations put forth in this report are primarily based on findings from two focus group discussions with patients, and one roundtable discussion with stakeholders. Hence, the recommendations were influenced by their input, which inadvertently skews towards a patient-centric view.

In the short-term, Malaysia should enhance its healthcare resource capacity (Pillar 1) through collaborative capacity building, resource sharing and the creation of a patient navigation platform to link healthcare facilities with various stakeholders.

In the mid-term, Malaysia should seek to create a comprehensive and sustainable framework for rare disease financing (Pillar 2) through legislated incentives and negotiated cost containment. As many of the solutions require interministerial collaboration, the governance system should be strengthened in order to achieve a holistic management of rare diseases (Pillar 3).

Lastly, in the long-term, the country's social protection systems (Pillar 4) need to be enhanced to empower and facilitate the resilience of individuals with rare diseases along with their caregivers. The implementation of these initiatives requires strong commitment, political will, and a driven champion to bring the necessary changes to fruition.

Strategic Pillars	Aspects of Policy Development	Timeframe
 <b>Pillar 1: Enhancing healthcare resources capacity</b>	<p>Building the capability of healthcare professionals through collaboration, strengthening the role of universities and prioritising government position warrants</p> <p>Resource sharing for diagnostic tests between interministerial facilities</p> <p>Improving seamless collaboration and integration between healthcare facilities and patient groups</p>	<p>Short-term <i>(1-2 years)</i></p>
 <b>Pillar 2: Comprehensive and sustainable rare disease financing</b>	<p>Cultivating the orphan drugs market through regulatory incentives</p> <p>Ensuring affordability of treatment through risk sharing mechanisms</p> <p>Leveraging pooled procurement to boost purchasing power</p> <p>Accelerating the Health Technological Assessment (HTA) process through joint assessment</p> <p>Strengthening the role of the trust fund to address unmet needs of rare disease treatment affordability</p> <p>Developing the pharmaceutical industry to promote generic competition for drugs treating rare diseases</p>	<p>Mid-term <i>(3-4 years)</i></p>
 <b>Pillar 3: Strengthening governance for holistic management of rare diseases</b>	<p>Institutionalising a legal framework for rare diseases to protect patients from heightened vulnerability</p> <p>Interministerial coordination and collaboration as key to improving integration and continuity of care</p>	
 <b>Pillar 4: Enhancing social protection systems to promote resilience of individuals with rare diseases and their caregivers</b>	<p>Protecting rare disease patients and householders with improved insurance coverage</p> <p>Improved coverage and assistance for support services and assistive devices</p> <p>Promoting inclusive employment opportunities for people living with rare diseases and their caregivers</p>	<p>Long-term <i>(more than 5 years)</i></p>