



UNDERSTANDING THE NATIONAL POLICY FOR RARE DISEASES IN MALAYSIA

This fact sheet provides a summary of the **National Policy for Rare Diseases in Malaysia**, developed by the Ministry of Health. It is intended for patients, families, the public, and healthcare professionals to understand the policy's goals and impact.

What is a Rare Disease in Malaysia?

A rare disease is a life-threatening or chronically debilitating condition. In Malaysia, a disease is officially defined as **rare** if it affects **fewer than 1 in 4,000 people**.

While individually uncommon, there are thousands of rare diseases that collectively affect a significant number of Malaysians. About 80% of these are genetic in origin.

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Our Vision & Mission

Vision

To improve the quality of life for individuals with rare diseases by ensuring they have fair access to healthcare and social welfare, creating an inclusive and supportive environment for all.



Mission

To build a healthcare system that provides early diagnosis, affordable treatment, and integrated support for patients and their families, upholding the principle of Universal Health Coverage.



This policy is the first-of-its-kind national framework designed to systematically address these issues.

The 9 Pillars of Action

The policy is built on nine key pillars to create a comprehensive support system:

Clinical Management:

Establishing centers of excellence, developing clinical practice guidelines, and ensuring patients receive timely, expert care.

01



02

Orphan Medicines:

Facilitating access to and registration of essential medicines for rare diseases.



Special Purpose Food:

Ensuring the availability and regulation of specialized medical nutrition.

03



04

Funding & Reimbursement:

Creating sustainable funding through a **National Rare Disease Trust Fund** and a clear process for treatment reimbursement.



Laboratory Services:

Expanding access to advanced diagnostic testing to shorten the time to diagnosis.

05



06

Advocacy & Health Education:

Raising awareness among the public and training healthcare professionals.



National Registry:

Establishing a national database to collect crucial data on rare diseases in Malaysia to inform policy, healthcare planning, and research.

07



08

Rehabilitative, Supportive & Social Care:

Providing integrated access to physiotherapy, occupational therapy, genetic counselling, psychological support, and palliative care.



Health Technology Assessment (HTA):

Creating a fair and transparent process to evaluate and approve new technologies and treatments for rare diseases.

09



For Patients and Families:

- Faster Diagnosis
- Better Access to Treatment
- Access to Financial Assistance
- Coordinated Care
- A Stronger Voice



For the Public:

- A More Inclusive Society
- Increased Awareness



For Healthcare Professionals:

- Clear Guidance
- Enhanced Training
- Research Opportunities
- Streamlined Processes



What This Policy Means for You