



MINISTRY OF HEALTH MALAYSIA

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.

Diagnostic Odyssey

It can take years to get an accurate diagnosis.

Need for Data

The absence of a national registry has made it difficult to plan services and conduct research.

01

05

Lack of Awareness

Low public and professional awareness can lead to delays in care and support.

Why This Policy is a Milestone

Living with a rare disease in Malaysia presents many challenges:

04

03

High Cost

Treatments known as orphan medicines, and special purpose foods can be extremely expensive.

Limited Treatment

Only about 5% of rare diseases have an approved treatment.

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



Special Purpose Food:

Ensuring the availability and regulation of specialized medical nutrition.

03



Laboratory Services:

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

05



National Registry:

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

07



Health Technology Assessment (HTA):

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

09



02



Orphan Medicines:

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

04



Funding & Reimbursement:

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

06



Advocacy & Health Education:

Raising awareness among the public and training healthcare professionals.

08



Rehabilitative, Supportive & Social Care:

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

What This Policy Means for You

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