FREQUENTLY ASKED QUESTIONS

NATIONAL POLICY FOR RARE DISEASES IN MALAYSIA

01 WHAT IS THE MAIN PURPOSE OF THE NATIONAL POLICY FOR RARE DISEASES IN MALAYSIA?

The main purpose of this policy is to improve the quality of life and promote the social and economic inclusion of individuals affected by rare diseases. It aims to remove obstacles to healthcare and social welfare services, creating an integrated support system that promotes access, equitable and sustainable healthcare system that guarantees early diagnosis, affordable treatment, and comprehensive support for individuals and families with rare diseases.

02 WHO DEVELOPED THIS POLICY?

The policy was developed by a multidisciplinary team from the Ministry of Health, universities, other ministries and agencies, as well as representatives of Patient Advocacy Groups, in collaboration with the Obstetrics & Gynaecological and Paediatric Services Unit of the Medical Development Division, Ministry of Health Malaysia.

03 WHAT ARE THE CORE PRINCIPLES OF THIS POLICY?

The policy is founded on several key principles:

- Acknowledging the severity of the problem
- Promoting national strategies and actions
- Advocating for the availability and affordability of medicines
- Developing synergies across different stakeholders and international borders

04 HOW DOES MALAYSIA OFFICIALLY DEFINE A "RARE DISEASE"?

Previously, Malaysia had no official definition for a rare disease. After consulting with various stakeholders, the Ministry of Health has defined rare disease as

"Rare disease is defined as a life-threatening and/or chronically debilitating rare condition that affects less than 1 in 4,000 people in the general population as listed in the Malaysian Rare Disease List"

05 IS THERE AN OFFICIAL LIST OF RARE DISEASES IN MALAYSIA?

A Malaysian Rare Disease List has been developed and will be updated periodically. The latest version is available through the official Ministry of Health (MOH) portal. (Penerbitan » Klinikal » Perkhidmatan O&G dan Pediatrik » Genetik/RD)

06 WHAT ARE THE CRITERIA FOR A DISEASE TO BE INCLUDED IN THE MALAYSIAN RARE DISEASE LIST?

A disease must meet the following criteria for inclusion

- There are confirmed patients in Malaysia
- The disease affects fewer than 1 in 4,000 people in Malaysia
- The disease is a severe condition
- Its inclusion is approved by the National Rare Disease Committee

07 HOW CAN ONE APPLY TO ADD A NEW DISEASE TO THE LIST?

A proposal form must be submitted by a registered medical practitioner to the Chairman of the National Rare Disease Committee. The proposal must include justification and supporting data for the inclusion criteria. The application is then screened by the secretariat and reviewed by a technical committee before being sent for final approval.

08 IS THERE A NATIONAL REGISTRY FOR RARE DISEASE PATIENTS IN MALAYSIA?

At the moment there is no national registry for rare diseases in place. However, one of the key components highlighted in the Policy is the establishment of a registry, which will serve as a foundation for the development of a National Rare Disease Registry. This registry is vital for collecting comprehensive patient data to improve diagnosis, facilitate research, establish disease prevalence, service planning, and support evidence-based health policies.

09 WHAT ARE THE KEY REFERRAL CENTRES FOR RARE DISEASES IN MALAYSIA?

Key referral centres include:

- Hospital Kuala Lumpur (national referral centre for rare diseases)
- Hospital Pulau Pinang
- University Malaya Medical Centre (UMMC)
- Hospital Universiti Sains Malaysia (HUSM)
- Hospital Pakar Kanak-Kanak UKM (HPKK UKM).

10 WHAT TYPES OF TREATMENT AND SUPPORT ARE AVAILABLE FOR PATIENTS?

The Ministry of Health provides various forms of care based on patient needs, including:

- Special formula milk
- Special medications known as Orphan Medicine
- Diagnostic tests and supportive treatments
- Rehabilitation services such as physiotherapy, occupational therapy, and palliative care

11 WHAT DOES THE POLICY SAY ABOUT EARLY DIAGNOSIS?

Early diagnosis is considered critical to allow for timely intervention. The policy acknowledges that many individuals endure lengthy "diagnostic odysseys" and advocates for timely access to diagnostic tests.

12 WHAT REHABILITATIVE AND SOCIAL CARE SERVICES ARE OUTLINED IN THE POLICY?

The policy emphasizes the importance of a wide range of services to improve quality of life. These include:

Early Intervention:

- A multidisciplinary team including physiotherapists, occupational therapists, speech therapists, dieticians, and clinical psychologists should develop individualized plans.

Social and Financial Support:

- Medical social workers assist families with financial aid, support groups, and other community resources.

Community Integration:

- Ensuring children have access to inclusive education and that adults are supported with vocational rehabilitation for employment.

Respite and Palliative Care:

- Providing temporary relief for caregivers and essential end-of-life care to manage symptoms and support families.

13 HOW ARE TREATMENTS FOR RARE DISEASES FUNDED?

Funding is a major challenge due to the high cost of treatment. The current mechanisms include:

- Government funding from the Ministry of Health
- Rare Disease Trust Fund
- Co-funding and full funding from corporate bodies, non-governmental organizations (NGOs), and government-linked companies
- Compassionate use by pharmaceutical companies

14 HAS A SPECIFIC FUND BEEN ESTABLISHED FOR RARE DISEASES?

Yes, the Rare Disease Trust Fund was established in 2021 at Hospital Tunku Azizah, Kuala Lumpur, to serve as a centralized resource for research, diagnosis, treatment, and support services. The Fund enables public contributions to support patients with rare diseases. The selection of patients eligible for assistance is guided by a clear framework established by the Ministry of Health, ensuring fairness and transparency. The implementation of this Policy will further strengthen and expand the role of the Trust Fund in meeting the needs of patients with rare diseases.

15 HOW DO WE GET MORE INFORMATION ABOUT THE TRUST FUND?

For further enquiries please email to the official email adress of the trust fund at akaunamanahpjj@moh.gov.my.

16 WHAT ARE "ORPHAN MEDICINES" AND HOW ARE THEY REGULATED?

Orphan medicine is defined as a medicinal product that is primarily intended to treat, prevent, or diagnose a rare disease as per the Malaysian Orphan Medicines Guideline, published by the Pharmaceutical Services Programme, Ministry of Health Malaysia. This guideline can be accessed via www.pharmacy.gov.my (Document & Media » Documents » Garis Panduan).

17 WHAT ABOUT "SPECIAL PURPOSE FOOD"?

"Special purpose food" refers to food formulated for individuals with specific nutritional requirements. For example, certain inborn errors of metabolism may require low protein diets, amino acid modified formulas, or other specialized nutritional products to support their medical management.

These products are regulated under the Food Regulations 1985. While they do not require pre market approval, manufacturers must comply with strict compositional and labelling requirements to ensure safety and suitability for their intended use.

18 HOW DOES THE POLICY SUPPORT RESEARCH ON RARE DISEASES?

The policy encourages research collaborations among clinicians, researchers, academic institutions, industry partners, and patient support groups. It also calls for the development of a rare disease research network to promote local research.

19 WHAT IS BEING DONE TO RAISE PUBLIC AWARENESS?

The Ministry of Health, along with NGOs, will enhance the awareness through media campaigns and educational modules targeting healthcare staff, the public, and specific demographics like women of reproductive age.

20 WHAT ARE THE NEXT STEPS AND EXPECTED ACTIONS FOLLOWING THE PUBLICATION OF THE NATIONAL RARE DISEASE POLICY?

Following the publication of the National Rare Disease Policy, the Ministry of Health (MOH) will focus on developing the accompanying Action Plan. This Action Plan will outline specific strategies, execution steps, timelines, and monitoring mechanisms to ensure that the policy is effectively translated into action. In short, the policy provides the direction, while the Action Plan will drive execution and ensure measurable outcomes for the rare disease community in Malaysia.

21 WHAT IS THE ROLE OF THE OTHER MINISTRIES & AGENCIES, IN THE IMPLEMENTATION OF THIS POLICY?

Other ministries and agencies involved in the development of this Policy will support its implementation through advocacy, awareness, and educational initiatives, helping to promote early identification, social support, and inclusion for individuals living with rare diseases.

22 WILL THERE BE A DEDICATED TASK FORCE OR NATIONAL COMMITTEE RESPONSIBLE FOR OVERSEEING THE IMPLEMENTATION AND PERIODIC REVIEW OF THE POLICY?

The National Rare Disease Committee, established in 2019, will oversee the implementation of this Policy, including ongoing efforts and periodic reviews.

23 FOR FURTHER QUERIES ON THE POLICY, WHO CAN WE CONTACT TO FIND OUT MORE?

For further queries regarding the Policy, stakeholders may contact the Secretariat of the National Rare Disease Committee at the Ministry of Health for information and assistance at upogpkkm@moh.gov.my

24 WITH THE POLICY COMPLETED, CAN WE EXPECT MALAYSIA TO HAVE A CLEARER INNOVATIVE FUNDING PATHWAY FOR ORPHAN MEDICINES, BREAKTHROUGHS AND THERAPEUTICS?

MOH has been engaging with various stakeholders on innovative funding models even prior to the development of the Policy, in line with advancements in technology and emerging treatment modalities. The Policy will guide the exploration of funding pathways for orphan medicines, new therapeutics, and breakthrough treatments for rare diseases.

25 WILL THIS POLICY HELP INDIVIDUALS OR FAMILIES AFFECTED BY RARE DISEASES ACCESS WELFARE ASSISTANCE, AND HOW IS MOH WORKING WITH SOCIAL WELFARE AGENCIES TO SUPPORT THEM?

The Policy itself does not automatically grant welfare assistance, but it strengthens coordination between MOH and relevant social agencies to facilitate access to existing support services and ensure better advocacy for individuals and families affected by rare diseases.

26 HOW OFTEN WILL THIS POLICY BE REVIEWED?

The Policy will be reviewed periodically by the National Rare Disease Committee to ensure its strategies remain relevant and responsive to evolving needs and developments in rare disease management.

27 WILL ACCESS TO TREATMENT OR MEDICATION BE EASIER AFTER THIS POLICY IS IMPLEMENTED?

The Policy aims to improve coordination, advocacy, and planning for rare disease services, which is expected to facilitate better access to treatment and medications over time, subject to clinical and regulatory considerations.

28 CAN I APPLY FOR ANY FINANCIAL ASSISTANCE THROUGH THIS POLICY?

The Policy does not provide direct financial assistance, but it supports coordination with relevant agencies to help individuals and families' access existing financial aid and social support programs.

29 WILL PATIENT ORGANISATIONS CONTINUE TO BE INVOLVED IN THE IMPLEMENTATION?

Yes, patient organisations will continue to play an important role in the Policy's implementation through advocacy, awareness, and collaboration with the Ministry of Health and other stakeholders.

30 HOW CAN MEMBERS OF THE PUBLIC CONTRIBUTE TO OR SUPPORT THIS POLICY?

Members of the public can support this Policy by participating in awareness initiatives, advocating for early detection and care, collaborating with patient organisations and healthcare providers to improve outcomes for the rare disease community and promoting contributions to the dedicated trust fund.

31 WILL THE GOVERNMENT LAUNCH PUBLIC AWARENESS CAMPAIGNS ABOUT RARE DISEASES?

Yes, the government will conduct ongoing public awareness and education campaigns on rare diseases to promote early detection, reduce stigma, and encourage community support.