IDEAS
INSTITUTE FOR DEMOCRACY AND ECONOMIC AFFAIRS

Financing Access to High-Cost Therapies for Rare Diseases in Malaysia:

A Practical Framework for Action with SMA as a Case Study

80%
rare diseases are genetic

500+
gene therapies in global development

How can Malaysia's health system sustainably finance & deliver ultra-high-cost, transformative therapies?

- Gene therapies cost millions per patient
- Procurement and finance lag behind medical innovation
- Risk that treatment access remains limited to the wealthy

Spinal Muscular Atrophy (SMA) in Malaysia

- Rare genetic neuromuscular disorder
- Early-onset forms (Type 1) most commonly present in infancy are associated with early mortality
- 1 in 20,000 to 50 born annually

Why SMA as the focus

- First condition in Malaysia with an approved gene therapy
- Entry point as pilot for future rare disease treatments

Outcomes-Based Payment Model (OBPM)

Contractual procurement mechanism where payments are linked to measurable patient outcomes over time

Is OBPM feasible within the MOH framework to ensure equitable access to high-cost treatments?

Priority Eligibility for OBPM

- High risk cases expedited
- Confirmed molecular diagnosis for <12 months
- <3 SMN2 gene copy number
- No life threatening symptoms
- Prioritize high motor CHOP score
- Pre-symptomatic
- Not on ventilator > 12 hours
- Not on invasive ventilator
- Below anti-drug antibody threshold

System Barriers Identified:

- **No newborn screening**
- **Eligibility exclusions inequitable**
- **Delayed diagnosis**

Policy recommendation:

MOH-led, tiered eligibility framework prioritising early diagnosis, expand newborn screening and diagnostics.

Outcomes That Trigger Payment

5-year survival

Motor gains (CHOP-INTEND)

Minimal complications (scoliosis, joint, feeding, pneumonia)

Not on ventilator support

- Assessed by independent committee
- Payments adjusted where outcomes are partially met or complications occur

Policy recommendation:

Expand outcomes beyond clinical metrics to include functional outcomes informed by patient and family needs.

Governance and System Readiness Gaps

- Coordination challenges (e.g. MOH-MOF)
- Annual budget not encompassing many rare diseases
- 6–9 month procurement timelines limit innovative contracting
- Varying SMA awareness, infrastructure in hospitals

Priority Room for Reforms

- Set up independent committee
- Clear national procurement guidelines
- Develop multi-year budgeting and payment frameworks
- Joint ASEAN procurement
- Develop national registries
- Strengthen advocacy ecosystems and infrastructure nationwide
- Improve transparency and communication

Beyond OBPM

Predictable outcomes for a treatment? **Consider pure financial payment stream**

Spread upfront costs across 15-20 years

Lower admin costs

Improve fiscal planning

Address ethical concerns around rigid outcome-linking & exclusion

Time-limited | Renegotiable | Cohort-based
| Avoid long-term supplier lock-in
| Flexibility from future competition and price reduction

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