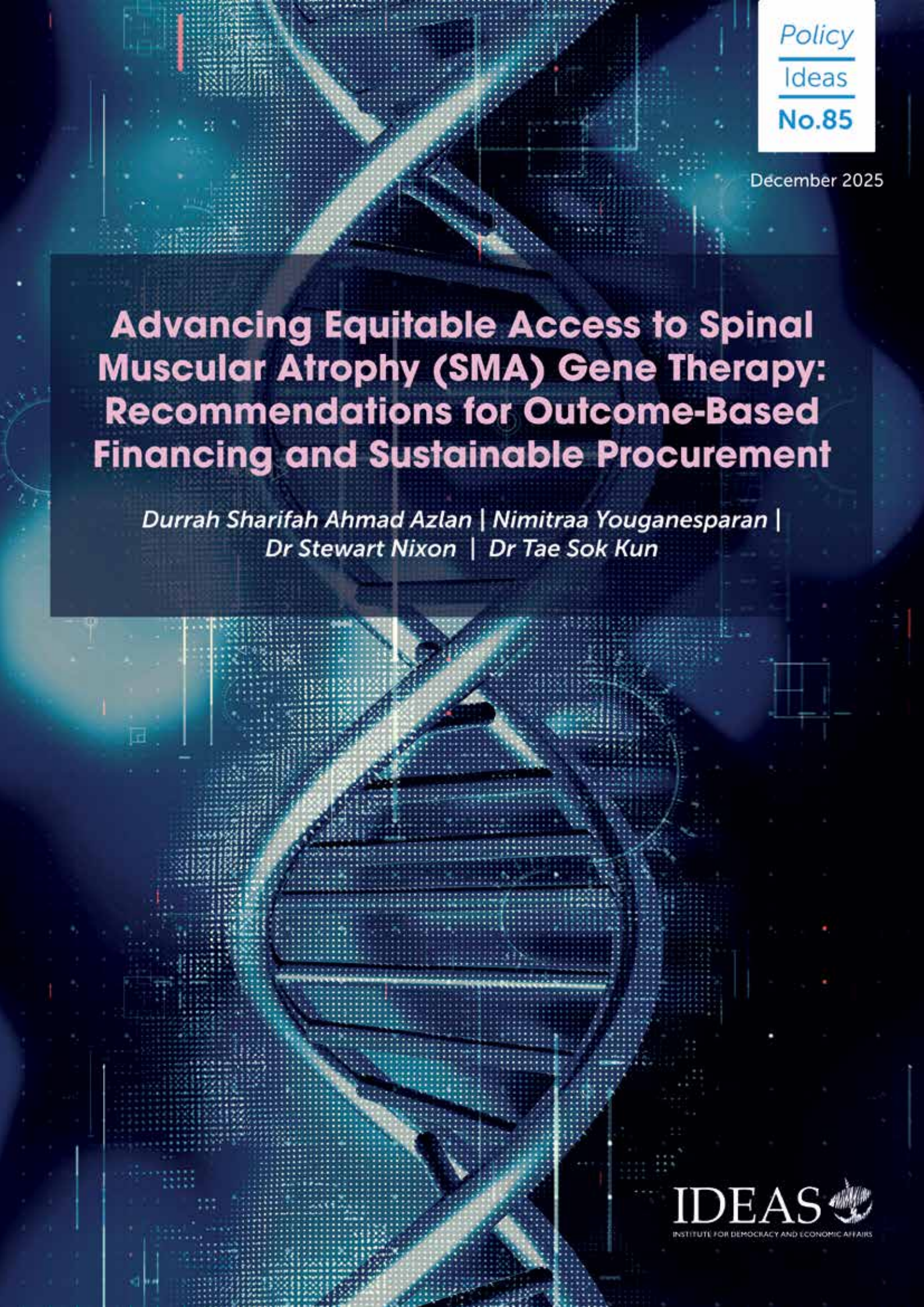

Policy Ideas No.85

December 2025

Advancing Equitable Access to Spinal Muscular Atrophy (SMA) Gene Therapy: Recommendations for Outcome-Based Financing and Sustainable Procurement

Durrah Sharifah Ahmad Azlan | Nimitraa Youganesparan | Dr Stewart Nixon | Dr Tae Sok Kun

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First published in December 2025

Published by:
IDEAS Policy Research Berhad (IDEAS)
The Lower Penthouse, Wisma Hang Sam, 1, Jalan Hang Lekir 50000 Kuala Lumpur

www.ideas.org.my
Reg No.: 1219187-V

Design and layout: Norhayati Murni Misran
Cover downloaded from: https://www.freepik.com

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Acknowledgements

This paper was developed by IDEAS independently with input from multiple stakeholders, including representatives from the Ministry of Health (MOH), clinicians, academicians, health organisations and think tank representatives, and patient advocacy groups. Specifically, we would like to thank these representatives and bodies, and also other individuals who prefer to remain anonymous for their participation and valuable insights:

- Spinal Muscular Atrophy Malaysia
- Malaysian Rare Disorders Society
- Dato' Dr Nor Fariza Ngah (Deputy Director-General of Health, Research and Technical Support, Ministry of Health Malaysia)
- Medical Development Division, Ministry of Health Malaysia
- Professor Datuk Dr Lokman Hakim bin Sulaiman (Deputy Vice Chancellor (Research), and Professor of Public Health, IMU University)
- Dr. Aliza binti Alias (Senior Principal Assistant Director, Pharmacy Practice & Development Division, Ministry of Health Malaysia)
- Professor Dr. Zilfalil Alwi (Senior Consultant Pediatrician & Clinical Geneticist, Professor of Medical Genetics, Universiti Sains Malaysia)
- Dr. Ngu Lock Hock (Consultant Clinical Geneticist, Pediatrician, and Head of Department for Genetic Clinic, Hospital Kuala Lumpur)
- Professor Thong Meow-Keong (Professor of Paediatrics and Consultant Clinical Geneticist, Universiti Malaya and Universiti Tunku Abdul Rahman)
- Associate Professor Dr Teguh Haryo Sasongko (Deputy Director (Consultancy, Laboratory Services, and Business Development), Institute for Research, Development, and Innovations, IMU University)
- Dr. Aina Mariana binti Abdul Manaf (Deputy Head of Unit, Paediatric Unit, Hospital Tuanku Jaafar)
- Charlene Tay Szu Lynn (Senior Principal Assistant Director, National Pharmaceutical Regulatory Agency, Ministry of Health Malaysia)
- Associate Professor Dr. Nazefah Abdul Hamid (Associate Professor, Faculty of Medicine and Health Sciences, Universiti Sains Islam Malaysia (USIM))
- Dr. Saw Pui San (Ministry of Health Research Collaborator and Discipline Head in Pharmacy Practice, Monash University Malaysia)

The authors would like to thank Aira Azhari, Alissa Rode, and Amalina Annuar for supervising this project. We would also like to thank Faiz Zaidi for his support in project management, Zuhdi Farhan, Ryan Panicker, and Wan Adam in event management and communications, and Norhayati Murni Misran for the design of this publication. We would also like to thank our interns, Nurazmina Dani, Siti Nur Atikah, and Iman Suhailan who have assisted in this report.

This project was funded by Novartis Malaysia and undertaken independently by IDEAS' researchers and fellows. The publication of this paper does not constitute or imply endorsement of any specific drug, product, company, or policy, unless explicitly stated otherwise by the respective party.

Executive Summary

Individuals impacted by rare diseases face systemic barriers to accessing emerging treatments, especially one-off, innovative therapies like gene therapy for Spinal Muscular Atrophy (SMA). The high upfront costs of these treatments are far beyond the reach of Malaysian households and pose a significant fiscal challenge for finite government health budgets. While Malaysian policymakers and health system stakeholders have made regulatory and policy progress towards advancing support for rare disease treatment, the necessary financing mechanisms, procurement rules, and clinical infrastructure have not developed concurrently. The inaccessibility of new treatments for rare diseases detracts from Malaysia's provision of equitable healthcare based on clinical need, not private wealth or external goodwill. To overcome this, innovative financing and procurement reform are essential.

This IDEAS policy paper explores policies to address clinical, procurement, financial and systemic barriers to providing sustainable and equitable access to high-cost SMA treatments in Malaysia. The objective was to explore the feasibility of implementing innovative financing models such as Outcome-Based Payment Models (OBPM) within the MOH's Medicine Access Schemes (MASc) framework and co-develop a practical model that is fiscally responsible and contextually relevant. This paper was developed in consultation with a diverse range of stakeholders including MOH officials, clinicians, patient caregivers, insurance representatives, and health economists.

The paper adopts a multi-method approach including comparative international benchmarking, qualitative focus groups (FGDs) with public health officials and specialists, and key informant interviews (KIIs) with patients/caregivers and financial sector experts. The resulting thematic analysis grounds policy recommendations in both clinical and lived experience.

The core findings advance a three-pronged strategy focusing on Clinical Readiness, Financing Innovation, and Systemic Governance.

Clinical Readiness and Patient-Centric Outcomes:

To ensure fairness and accountability, the paper recommends establishing clear, localised eligibility criteria based on clinical consensus (e.g., confirmed diagnosis, and specific SMN2 gene copy number) while also acknowledging patient community concerns about overly restrictive access. For assessing treatment efficacy, establishing outcome criteria that moves beyond motor function scores to incorporate Quality of Life (QoL) indicators, caregiver satisfaction, and the achievement of stabilisation against progressive deterioration is recommended. In addition, strengthening system-wide readiness through the implementation of Newborn Screening (NBS) and establishment of a National Rare Disease Registry would further support clinically targeted eligibility, allowing for more equitable access, informed decision-making, and long-term evaluation of treatment impact.

Procurement and Financing Innovation:

While the principles underpinning OBPM are valuable for understanding treatment risk, for established treatments with well established outcomes such as gene therapy for SMA, the cost and administrative burden of linking payments to outcomes is not justifiable. Therefore, for SMA gene therapy, the paper recommends pursuing a pure financial payment stream to spread the high upfront payment over multiple years (15–20 years). This approach dramatically reduces the annual fiscal commitment for the government, making procurement more manageable within existing budgets, without compromising efficiency. OBPM nonetheless has potential applications for future high-uncertainty, high-cost therapies and would benefit from a trial application involving a treatment meeting these characteristics.

Systemic and Ethical Governance:

Meaningful and lasting reform requires systemic uplift across the health, financial, and ethical ecosystem. Recommendations include:

- Establishing a dedicated process for assessing and publishing the comprehensive financial profile (total costs, patient numbers, long-term burden) of emerging treatments. The process would be led by MOH and access would be voluntary, operated on the basis of cost recovery (via a fee levied on the treatment provider), and subject to legislated timeliness and quality standards.

- Recognise that sustainably financing rare disease treatments requires a mature risk pooling system to leverage economies of scale. Achieving this requires significant medium-to-long-term reforms to deepen Malaysia's health insurance market and establish a National Medical Insurance Fund.

- With a mature insurance market or National Medical Insurance Fund, inclusive coverage could be affordably regulated to incorporate approved rare disease treatments as default inclusions in medical insurance policies. Mandatory default coverage is the only plausible pathway to providing insurance for early onset genetic disorders like SMA.

- Embedding ethical governance to ensure transparency, address the profound financial and emotional toll experienced by patients and caregivers (as detailed in the KIIs), and manage sensitive data sharing within any financing model.

This paper serves as a practical roadmap for policymakers, demonstrating that sustainable access to transformative treatments like SMA gene therapy is fiscally attainable through strategic procurement and financial innovation. By addressing these foundational clinical, financial, and governance barriers, Malaysia can institutionalise value-based procurement for future transformative treatments, ensuring equitable access for all citizens affected by rare diseases.

1.0 Introduction

Individuals with rare diseases face systemic barriers to accessing emerging treatments in Malaysia, especially one-off, innovative therapies. This paper explores the case of SMA and innovative gene therapies to treat it, as well as how health system shortcomings can be overcome to make treatment more accessible and equitable.

Despite Malaysia's commitment to advancing health access, the necessary financing mechanisms, procurement rules, and clinical infrastructure have not developed concurrently with the approval of groundbreaking treatments. This risks Malaysia falling behind in the global gene therapy race and perpetuating inequities, as access remains largely dependent on private wealth or external goodwill. To overcome this impasse, innovative financing models that align costs with value and leverage pooled funding are essential.

Objective

This paper, spearheaded by IDEAS and developed in consultation with multiple stakeholders, addresses the urgent need to secure sustainable and equitable access to high-cost SMA treatments within Malaysia. It was guided by the specific objective to explore the feasibility of implementing OBPMs within the Ministry of Health's existing Medicine Access Schemes (MASc) framework. The aim is to co-develop a practical financing and procurement model that is contextually relevant, transparent, and fiscally responsible.

Methods

The paper employs a rigorous, multi-method approach:

- Comparative analysis and international benchmarking: A preliminary review of local and global evidence, assessing international models of OBPMs (e.g., in Brazil and Italy) for cell and gene therapy treatments to define best practices for eligibility, outcome milestones, and implementation protocols.

- Stakeholder engagement: Structured Focus Group Discussions (FGDs) that included 19 participants, among them senior MOH officials, clinical specialists (paediatrics, neurology, genetics), and public health experts. Key Informant Interviews (KIIs) were conducted with parents/caregivers of SMA patients, insurance representatives, and health economists to integrate patient and financial ecosystem perspectives. The resulting qualitative data was subjected to a thematic analysis to generate evidence-based recommendations.

What will be covered

Building on these insights, the paper presents practical, targeted solutions to support policymakers. The remainder of this paper is structured as follows:

- The background provides contextual information on rare disease treatment and management in Malaysia, exploring the impediments to equitable access that create a need for innovative approaches.

- Sections that explore:

    - The eligibility criteria and outcome milestones required to guide clinical decision-making and ensure accountability.

    - Procurement and health economics/financing models and reforms to support sustainable and equitable access to treatment.

    - Complementary health systems and governance reforms to support the eligibility and procurement improvements.

Ultimately, this paper seeks to inform policy decisions that advance equitable access to SMA gene therapy, serving as a catalyst for institutionalising value-based procurement for future transformative treatments.

2.0 Background

> "After the difficult diagnosis, the second big crisis for SMA patients is the financial one. Because the medical treatment is not affordable... As I've mentioned earlier, I started to give up looking for medical treatment around my son's recent birthday. I decided, whatever happens, happens."
> ***— Parent of child afflicted with SMA Type 1***

Individuals impacted by rare diseases face systemic barriers to accessing emerging treatments, especially one-off innovative therapies. For context, the MOH defines rare diseases as a life-threatening and/or chronically debilitating rare condition affecting less than 1 in 4,000 people (MOH, 2020). Rare diseases are complex, present differently for each patient, and a lack of awareness among healthcare professionals and steep financial costs prevent treatments from being widely accessible.

SMA is a rare disease that showcases these challenges. SMA is a genetic neuromuscular disorder that results in progressive muscle weakness and atrophy, encompassing a clinical continuum characterised by varying degrees of severity and age of onset (Abu Hussain et al., 2024). SMA Type 1 represents the most severe form, typically manifesting in early infancy with profound muscle weakness and respiratory compromise, often leading to early mortality if untreated. Type 2 SMA presents between six to 18 months of age and is characterised by significant motor delay and reduced lifespan, typically into adolescence. Type 3 SMA, or Kugelberg–Welander disease, has onset after 18 months and is associated with milder symptoms, allowing affected individuals to walk independently for several years before gradual decline. Type 4 SMA, the adult-onset form, usually manifests after the age of 21 and is associated with the mildest course and near-normal life expectancy (D'Amico et al., 2011).

The true number of Malaysians with SMA remains unknown due to the absence of a national SMA registry and limitations in diagnosis and data systems. While global incidence is estimated at approximately 1 in 10,000 live births (Lunn & Wang, 2008), Malaysia's reported estimate of 1 in 20,000 (Ch'ng et al., 2018) suggests either a genuinely lower incidence or, more likely, under-detection and under-reporting. Reflecting this uncertainty, only 20–30 cases were reported annually between 2013 and 2017 (Institute for Medical Research, 2019, p.11). However, this was prior to the introduction of newborn screening. Advocacy group WeCare Journey (2022) further estimated that around 50 babies are born annually with SMA, highlighting the urgent need for improved case identification and a national registry to establish accurate prevalence data.

Providing affordable and equitable medical care and opportunities for development is not only a policy goal but a right enshrined in international law. This includes Malaysia's commitment to the UN Convention on the Rights of the Child (UNCRC), which affirms the right of all children to non-discrimination, survival and development, and access to healthcare.

Yet, in Malaysia, children with rare diseases including SMA often face systemic inequities, including stigma, poverty, and exclusion from education and social services that compound the health-related barriers they experience. Addressing this requires a coordinated, whole-of-government response that extends beyond the Ministry of Health. Such efforts are also central to advancing Malaysia MADANI's principles of Care and Compassion (Ihsan) and Sustainability (Kemampanan), while also supporting the country's progress toward the Sustainable Development Goals, particularly Goal 3 (Good Health and Well-being) and Goal 10 (Reduced Inequalities).

Against this backdrop, the introduction of disease-modifying therapies for SMA offers hope for patients and their families for what used to be considered a life sentence. Globally, access to emerging treatments has significantly improved survival, motor function, and overall quality of life, especially when administered early in infancy (Thong et al., 2022). Administered either on a one-off (onasemnogene abeparvovec [OA]), (Zolgensma) or ongoing (nusinersen [Spinraza] and risdiplam [Everydi]) basis, these therapies work by compensating for the defective gene responsible for SMA, allowing for better muscle function and developmental outcomes (Gowda et al., 2024; U.S. Food and Drug Administration, 2025). In early 2024, Malaysia approved its first gene therapy for SMA, onasemnogene abeparvovec[1] (OA), marking an important milestone in expanding access to advanced treatments for rare diseases (CodeBlue, 2024).

In Malaysia, access to these therapies remains extremely limited due to their prohibitive costs and the absence of supporting infrastructure. The estimated annual treatment cost for Nusinersen — an injection that needs to be taken once every 4 months — is up to USD 708,000. Risdiplam — an oral drug with each bottle lasting for a month — costs approximately USD 354,000 a year (Canadian Agency for Drugs and Technologies in Health, 2022). Meanwhile, a one-off course of OA costs around USD 2 million (Nuijten, 2021). With payments ordinarily linked to treatment administration, one-time therapies like OA involve steep upfront costs for patients or health systems, as manufacturers recover costs in a single charge. Despite its high upfront cost, OA's single-dose regimen is seen as more cost-effective over a lifetime compared to other therapies that require ongoing administration (Ogbonmide et al., 2023). Compassionate care programmes have provided temporary access to treatments like OA for selected patients (Thong et al., 2022).

Beyond direct therapeutic costs, non-financial barriers such as delayed diagnosis, limited specialist capacity, and weak care coordination further constrain equitable access. This reflects that in Malaysia, an exclusive regulatory focus on product safety results in therapies being approved for use without the concurrent development of clinical criteria, financing models, and health system infrastructure necessary to ensure fair distribution. While safety is paramount, relative inattention to access considerations raises serious equity concerns in a resource-constrained setting such as Malaysia, where decisions on allocation directly impact which patients can benefit.

1. Onasemnogene abeparvovec is the scientific name for Zolgensma, an adeno-associated virus vector-based gene therapy that delivers a fully functional copy of human SMN gene into the target motor neuron cells.

Awareness of SMA remains limited in Malaysia, leading to delayed diagnoses and inadequate medical, financial, and pastoral support for patients and their families. Parents often report frustration and distress due to dismissive healthcare professionals, lack of information about the condition, and insufficient supportive services, including physiotherapy (Ch'ng et al., 2022). Genetic counselling is also a gap with delayed, urban-centric services due to limited capacity. This gap in awareness and support exacerbates the physical, emotional, and financial strain experienced by families. Financial challenges are particularly severe, as raising a child with SMA entails high costs for medical equipment, supportive devices, home modifications, and additional care services. Moreover, health insurance coverage for rare (especially genetic) diseases in Malaysia is minimal — both in terms of people (few pre-birth policies) and expenses (exclusions for emerging treatments) — pushing families to seek charitable assistance, crowd-sourced funding, or expensive personal loans. Box 1 illustrates the struggles faced, as well as quotes from parents of patients with SMA (see table 2 in appendix for coding) and their personal experiences in financing treatments.

Box 1: Living with SMA in Malaysia: The High Cost of Hope

Interviews with patients and caregivers revealed the extent of the sacrifices made and the challenges they face. Little to no support is provided to patients and caregivers in their efforts to seek treatment and care services. While some institutional and government support exists for SMA patients in Malaysia, caregivers still face significant financial burdens due to the extremely high and often unstable cost of medication. From a financial perspective, support in the form of subsidies and government aid from agencies such as the Public Service Department (JPA) and MOH, as well as aid from patient groups, reduces treatment costs significantly, yet the costs of care beyond medication are still a burden. Insurance plans are reluctant to cover rare diseases due to the limited information on the number of potential patients and high per patient healthcare costs. Medication costs are only one part of the equation, with indirect costs in the form of hospital bills and machine purchases (BiPAP machine) (S04, S06) making up a significant portion of spending for patients.

Crowdfunding is viewed as the most common method of financing high cost treatments, though its unsustainability is a common theme with caregivers. Surpassing more than 50% between 2019 and 2022 (Subashini, 2025), medical crowdfunding has gained popularity as the patients and families turn to public donations amid a lack of other finance options. In the case of SMA however, this method of financing treatments is seen as unsustainable and unrealistic, mainly due to the high cost of SMA medication. A common complaint was that unauthorised crowdfunding platforms often take a large cut of the donations, around 50% in some cases (S04), leaving caregivers with little left to finance the necessary treatments.

Caregivers were also left to bear the brunt of ignorance and negativity associated with crowdfunding on social media, receiving demeaning comments on posts questioning their intentions. This further deters them from turning to public crowdfunding in the future, *"there were comments online saying 'these people are lazy to work,' people don't understand."* (S07). Despite this, some caregivers feel there is no other option for financing these high cost treatments, having exhausted other means, *"because right now, aside from crowdfunding... we really don't have any other options, right?"* (S02).

The diagnosis of SMA irrevocably alters the lives of caregivers, necessitating significant trade-offs and life-changing decisions. In addition to having to make lifestyle changes to accommodate a family member who has been diagnosed, caregivers also experience immense psychological strain in handling SMA patient care. Parents expressed dread and anxiety over their child's lifespan and quality of life following diagnosis (S04 & S06), with many families opting to have at least one parent to stay at home. For some this means retiring early to tend to their children, as they are reluctant to leave that care to someone else (S01, S07 & S08).

The effects of SMA extend far beyond the medical aspect, permanently changing the lives of affected families. For both the patients and their caregivers, a robust social life is sacrificed for the sake of patient care, with other family members sidelined to instead focus on caring for the patient (S02 & S04). Outings and family visits are also often avoided due to risk of infection (S02 & S08). This adds to not only limited social interactions but also a sense of separation from the broader community, despite efforts by patient-group associations trying their best to engage with these families through community-centred events that involve the patients.

On top of that, even when SMA patients have achieved desired outcomes in their treatment, they often face significant barriers to achieving a fulfilling life, particularly in regards to their educational career and social life. Caregivers attempting to register their child for formal education received little to no support from the schools, with some institutions claiming insufficient facilities to handle special needs students (S05 & S06) as a reason to reject the enrolment of SMA patients. These experiences reflect the struggles faced by patients and caregivers that are often not highlighted.

Additional reflections from patients' and their families include:

"So far, most parents manage to pay through crowdfunding. My son's crowdfunding wasn't very successful. To date, he only managed to raise around RM36,000. Then, the platform provider took a cut—I'm not sure, but it cost around RM15,000. So I lost about 50% of the funds. I think I only had RM15,000–20,000 left. So I had to look for other alternatives. Crowdfunding doesn't always work." (S04)

"Yeah, I feel very helpless. And the helplessness is worse when it's sort of combined with people telling you hopelessness. Right? So, you're telling me to give up. You're telling me I've got one year left. You're telling me all these things. You're telling me to not do anything. You know, I cannot lie as a parent. I need to figure out what's reasonable, sensible to be done. Then, if it comes. You've just got to start from scratch. Reach out to as many people that can help you. To cope. And to figure things out. The first point of call would be families." (S06)

"I rely on my husband. His salary is below RM2,000... he also works with Grab and takes food orders as a side income...

..It's difficult. To even reach RM40,000 in how many months is extremely hard.

Applying for Zakat (Islamic charitable fund), special milk is applied for through Zakat.

All our lives, we constantly have to think about getting treatment. People don't understand the need for aid never stops, even now there's still help needed." (S07)

Innovative approaches and systemic policy reforms are needed for Malaysians to benefit from these and other transformative therapies. There is no single or simple solution, rather a strategic and comprehensive approach is needed that addresses clinical barriers and information gaps with sustainable financing mechanisms and procurement processes that prioritise equitable access. Policy reforms that support access to gene therapy for SMA patients are considered with a mind to broader applications across rare diseases and emerging therapies generally.

SMA serves as a practical entry point to understand the barriers and opportunities in financing and delivering high-cost, one-off therapies for rare diseases. The lessons from SMA can inform diagnosis, data systems, and financing mechanisms that can accommodate other rare and high-cost conditions. At the highest level, these insights can drive wider health system reforms involving procurement, promote sustainable financing, and embed equity at the centre of innovation policy. In this way, advancing access to gene therapy for SMA becomes a catalyst for ensuring Malaysia's health system is equipped to deliver emerging treatments more equitably and sustainably. Building on these insights, the next section examines Malaysia's evolving policy landscape for rare diseases and highlights opportunities for system strengthening that align with these objectives.

Evolving Policy Landscape for Rare Diseases in Malaysia

This paper builds upon previous IDEAS publications on rare diseases, which highlight general reform needs. The first such report in 2018 outlined the key challenges faced by rare disease patients in Malaysia, which include limited recognition, inadequate

healthcare access, and lack of legal and financial protection. It called for legislative reform, improved funding, and greater awareness (Thong & Ahmad-Annuar, 2018). This was followed by the publication of IDEAS' White Paper on Rare Diseases in Malaysia, and a policy brief on Improving Access to Orphan Drugs in Malaysia by Shafie (2019). These publications highlight gaps such as the lack of funding for healthcare services, the lack of accurate data on rare diseases, and the lack of a standardised definition of rare diseases in Malaysia. These efforts led to the successful inception of a National Rare Diseases Framework, and the formation of the National Rare Disease Committee by the MOH, Malaysia. Subsequently, IDEAS also published a report entitled "Next Steps for Rare Diseases in Malaysia: Improving Access to Treatments" in 2020, which explored the setting up of a trust fund and increased regional collaboration alongside various other policy recommendations (Todd & Rao, 2020).

From 2020 to 2024, government policies have modestly increased support for rare disease patients and their families without delivering major improvements. In 2021, MOH allocated RM10 million for the follow-up treatment of rare disease patients, and to begin treatment for patients on waitlists (CodeBlue, 2021). In the same year, MOH created a trust fund account to channel donations for the treatment of rare diseases (Ministry of Finance, 2022). The establishment of this trust fund marks an important milestone, reflecting the government's commitment to improving financial accessibility for rare disease patients.

In the past 15 months, the government signalled its appetite to provide additional support. The Ministry of Health Malaysia released the first ever National Policy on Rare Diseases in August 2025 (Medical Development Division, Ministry of Health, 2025), formalising Malaysia's strategic commitment to increasing access to treatment and management of rare diseases. Additionally, Budget 2025 increased the allocation to cover rare disease medical costs to RM25 million and expanded the MySalam scheme to include coverage for rare diseases (Ministry of Finance, 2024).

Momentum was further reflected in stakeholder engagement. The 2024 Malaysian Society for Pharmacoeconomics and Outcomes Research (MySPOR) Annual General Meeting brought together stakeholders from government, industry, academia, and patient groups to deliberate on financing mechanisms for high-cost therapies such as gene therapies. The discussions provided not just a platform to exchange knowledge and align priorities on managing financial and access barriers for rare diseases in Malaysia, but also expressed interest in outcome-based patient assistance schemes, which link payment to measurable clinical results rather than treatment administration alone. These discussions indicated the potential of OBPM to improve access to life-changing therapies while managing fiscal risk and ensuring accountability for health outcomes.

Malaysia's commitment to global priorities also became more visible in May 2025, when it joined a World Health Assembly resolution recognising rare diseases as a global public health priority (Rare Diseases International, 2025). This commitment adds urgency to Malaysia's need to improve access to high-cost treatments such as gene therapy, especially for conditions like SMA where patient benefits are well established.

Increasing political will provides a platform to make further progress towards accessible and equitable rare disease care. Future advancements should not only target immediate policy gaps but inspire a comprehensive longer-term system roadmap that integrates health and economic considerations. This includes targeting short-term quick wins such as exploring innovative financing models for high-cost therapies in addition to improving diagnosis pathways and patient registries, while also pursuing "moonshot" strategies such as insurance deepening. Ultimately, this paper contributes to that trajectory by outlining practical recommendations on how policymakers can do more to advance equitable access for patients with rare diseases in Malaysia.

Difficulties Encountered in Past Attempts to Finance High-Cost Therapies

Charting the course for policy uplift requires learning from earlier policymaker attempts to leverage financial innovation to fund emerging treatments. One such innovation is the Patient Access Scheme (PASc), which allows pharmaceutical companies to offer treatments at zero or reduced cost through structured schemes, either financial-based (unlinked to outcomes) or performance-based (tied to patient response). The scheme has benefited an estimated 12,000 patients from 2013 to 2016 (Boo, 2018).

However, the PASc scheme was suspended for a short while in 2018 following serious concerns about transparency and integrity in its implementation. The suspension came after then Director-General of Health, Tan Sri Dr. Noor Hisham Abdullah, raised ethical questions about how the scheme allowed pharmaceutical companies to supply medication while bypassing official procurement documentation (Kannan, 2018). Audits in 2019 vindicated this concern, revealing that MOH lost RM13.07 million from defective ventilators that could not be claimed due to procurement documentation deficiencies (CodeBlue, 2023). The scheme recommenced in early 2019, with a clear auditing mechanism in place (CodeBlue, 2019).

In an effort to formalise and bring greater oversight to medicine access schemes, the MOH published updated procedures in the second edition of the *Garis Panduan Pengendalian MASc di Fasiliti KKM* in March 2024 (MOH, 2024). It covers the end-to-end process: from application and approval, to procurement, storage, supply, and monitoring. The guideline consolidates three access mechanisms under one framework collectively known as the Medicine Access Schemes (MASc), which include:

1. **Sample Treatments:** Free drugs not tied to procurement, offered as samples. No claims can be made by patients should there be adverse reactions from the treatment.

2. **PAPSA:** Drugs donated for a fixed treatment duration, typically 6–12 months, for unregistered or unlisted medicines, to be used at specific MOH facilities. No claims can be made by patients should there be adverse reactions from the treatment.

3. **PASc:** For registered, formulary-listed drugs in Ministry of Health Medicines Formulary (MOHMF), which is divided into financial-based (most used) and performance-based (yet to be used) schemes.

Understanding how MASc is structured is key because it illustrates the different regulatory pathways that currently exist for accessing high-cost or unlisted therapies within the public system. It also highlights where policy and system gaps remain particularly around governance, accountability, and readiness to adopt these payment approaches.

While the updated guidelines are an important step forward in regulating medicine access schemes, they remain relatively new and untested. Notably, although PASc now includes guidance to accommodate both financial-based and performance-based models, only the financial-based model has been used to date. There has yet to be a formal proposal or implementation of an outcomes-based payment scheme.

This growing but still incomplete framework emphasises the need to expand equitable and affordable access to emerging, high-cost therapies (such as those for rare diseases) while ensuring transparency, accountability and patient safety as access schemes expand within Malaysia's public healthcare system, as echoed by stakeholders highlighted in the next section.

Previously Raised Stakeholder Concerns with Existing Procurement and Access Models

A 2022 qualitative study of PASc stakeholders including those from MOH, pharmaceutical firms, and advocacy groups, identified several key implementation barriers. These include administrative complexity, poor outcome data systems, legal uncertainty, and a lack of trust among stakeholders (Thanimalai et al., 2022). While financial-based schemes were seen as easier to implement, outcomes or performance-based schemes were considered more suitable for rare and novel diseases where the clinical benefit is uncertain and evidence generation is ongoing. In such cases, linking payment to demonstrated patient outcomes could help manage risks for both the payer and manufacturer while ensuring access for patients with limited treatment alternatives.

However, no OBPM had been successfully piloted in Malaysia at the time of the 2022 study, with some believing that it had a poor likelihood of being implemented. The main constraints included the absence of reliable outcome and finance data, the complexity of contract negotiations, limited legal frameworks to manage risk-sharing arrangements, and insufficient technical capacity within MOH. Participants emphasised several priorities for reform, including the need for government-led or co-led PASc initiatives for legitimacy and transparency, urgent data infrastructure improvements incorporating real-world evidence and digital monitoring tools, and the establishment of multidisciplinary PASc teams at MOH facilities to oversee implementation, governance, and evaluation. Without these structural reforms, current access mechanisms remain limited in scale, highly dependent on external goodwill, and vulnerable to administrative disruptions. Meanwhile, patient advocacy groups highlighted delayed diagnoses, financial strain, and fragmented service delivery as key barriers. These insights reinforce the need for a comprehensive, patient-centered, and equity-driven framework for high-cost therapy access.

OBPMs were revisited during the MySPOR 2024 meeting. Stakeholders explored OBPMs, risk-sharing agreements, and enhancements to MOH's procurement and HTA systems.

The meeting emphasised the leveraging of public-private partnerships and trust funds, strengthening MaHTAS to assess gene therapies using international HTA benchmarks, and addressing legal, data, and governance reforms needed to operationalise OBPMs within MASc.

Nevertheless, the aforementioned discussions were exploratory and involved a limited range of stakeholder perspectives. The design and implementation of OBPM inherently demands granular, cross-sector expertise spanning health, procurement, legal, and financial domains. As such, while the discussions provide valuable groundwork on systemic barriers, the operational complexities of integrating OBPMs into Malaysia's procurement ecosystem remain largely unaddressed. Without coordinated and timely policy action, Malaysia risks falling behind as other health systems move rapidly to adopt innovative financing models that enable access to cutting-edge treatments such as gene therapy.

Malaysia Risks Falling Behind in the Global Gene Therapy Race

Globally, gene therapy innovation is advancing at an unprecedented pace, offering superior health outcomes and less invasive treatments. As of May 2025, the U.S. Food and Drug Administration (2025) has approved 45 gene therapies, with 500 more in development and 10–20 expected approvals annually (Segal Group, 2024). These therapies, often designed as single-dose treatments for rare or genetic conditions, represent a major shift in how care is delivered.

The pace of innovation is matched only by the soaring price tags of these treatments. Since Zolgensma's debut as the world's most expensive drug in 2019, by August 2025 it had been overtaken by at least nine costlier gene therapies including Lenmeldy (USD 4.25 million), Kebilidi (USD 3.95 million), and Hemgenix (USD 3.50 million) (Becker et al., 2025).

While these innovations mark major therapeutic breakthroughs, their commercial adoption has often been slow, even in well-resourced systems. Several have reached only a handful of patients, and others have faced market withdrawals due to insufficient supporting infrastructure and unclear reimbursement pathways. Experience shows that policy readiness does not stop with therapeutic or safety approval, with broader health ecosystem gaps undermining adoption of innovative treatments.

The situation is particularly acute in Malaysia, where rapid advances in gene therapy are not being reflected in policy or patient treatment. Malaysia has approved only one gene therapy: Zolgensma for SMA in 2024. While other treatments are available provisionally under mutual recognition arrangements, their local use remains uncertain. The National Pharmaceutical Regulatory Agency, (NPRA)'s updated Guidance Document and Guidelines for Registration of Cell and Gene Therapy Products (CGTPs) (September 2025) sets clearer rules for product registration and oversight, yet translating such regulatory progress into patient access will depend on parallel advances in financing, procurement, and cross-agency coordination.

As more single-dose, high-impact therapies become available, Malaysia must move quickly to define suitable access pathways and financing models. As the first condition with an approved gene therapy, SMA serves as a practical entry point to explore how outcomes-linked financing can be operationalised within the broader health and procurement ecosystem by serving as a starting point for system-wide innovation in access and financing.

3.0 Purpose and methodology

This paper began with the central question: Would an OBPM support more equitable and affordable access to emerging gene therapy treatments for SMA patients in Malaysia? It further examined what clinical eligibility and outcomes criteria, what procurement and financing reforms, and what systemic changes across the health and financial ecosystem would be necessary to make such a model feasible.

To explore these questions, the paper employed a qualitative, multi-method approach to develop a policy framework for equitable access to gene therapy in Malaysia, with a specific focus on OBPM, using approved gene therapy for SMA as a case study. Keeping in mind the heavy administrative burden on clinicians and the absence of a standardised patient selection protocol, the project proposes to support MOH in developing a practical, evidence-based rubric in close consultation with policymakers from MOH.

The methodology included research identifying and assessing international best practices, stakeholder engagement through focus group discussions (FGDs), and key informant interviews with relevant government and procurement stakeholders. The FGDs and interviews brought together a diverse range of voices across the ecosystem including clinicians, academic researchers, policymakers, health economists, insurance representatives, and most importantly SMA patients and their caregivers. Participants were selected based on their expertise or direct experience with rare disease treatment, clinical management, or healthcare policy in Malaysia. Data collection activities, including FGDs and interviews, were conducted within the 2024–2025 period. While the primary policy application is Malaysia, the comparative research explored international experiences to assess the adaptability of financing strategies in middle to low income health systems. The report aims to bridge global policy innovation with the realities of Malaysia's public procurement and healthcare financing environment. Table 1 lists down the different types of data collection carried out, and the participants.

Table 1: Activities and participants

Activity	Participants
Focus Group Discussion An interactive group discussion was conducted on topics of interest that included eligibility and clinical criteria, outcome milestones, procurements, systems and governance.	19 participants, including senior officers and specialists from various divisions within the MOH, including Pharmacy Practice and Development, National Pharmaceutical Regulatory, Medical Development Division, and Pharmaceutical Policy and Strategic Planning. Also in attendance were consultant clinical geneticists, consultant paediatricians, public health experts and unit heads from leading hospitals and research institutes, as well as academic professionals from Malaysian universities with expertise in paediatrics, neurology, genetics, and medical research.
In-depth Interviews	10 interviewees, made up of parents and caregivers of SMA Type 1, Type 2 and Type 3 patients, an SMA Type 2 patient, insurance representatives and a health economist.

4.0 Findings

4.1 Eligibility Criteria

Malaysia needs to establish clear and context-sensitive eligibility criteria for rare disease treatments, including gene therapies. Localised criteria play a pivotal role in paving the way for equitable access to high cost therapies, guiding clinical decision-making, and informing recommendations that reflect national healthcare system priorities and capacity.

Equitable access is built upon clear eligibility criteria. Eligibility based on clinical need and appropriateness and supported by complete information for practitioners is essential to making emerging and high-cost therapies available to those that need them. In the absence of clear criteria, access might unfairly depend on non-clinical factors, such as location and the ability of patients to pay out of pocket, disproportionately affecting patients in more rural areas and those with less financial means. It also helps the ministry define a set patient group, make procurement decisions, and establish patient support needs by setting aside the necessary resources and services based on the needs of this particular group.

From a clinical standpoint, clear eligibility parameters remove key knowledge and risk barriers to prescribing high-cost therapies. Emerging treatments are a riskier treatment choice for clinicians and patients as they have lesser clinical certainty. Information, expertise and practical experience surrounding rare disease patients and treatment is also scarce. The limited and precise nature of gene therapies often causes confusion as to when and for whom they should be prescribed, even for experienced clinicians. This is especially relevant for SMA which ranges from Type 0 and Type 4, with varying severity and treatment response. Although all three disease-modifying therapies offer clinical benefits, their effectiveness varies greatly depending on factors such as the SMN2 copy number, age at treatment, and whether the patient is pre-symptomatic. In addition, it should be noted that not all therapies are approved for the different types of SMA presentations.

Gene therapies that deliver a working copy of the SMN1 gene were found to have marked improvements in infants with SMA, particularly when administered early (Pascual-Morena et al., 2022). Clinical criteria should be based on a combination of age, weight, molecular diagnosis, anti-AAV9 antibody titers, and other relevant clinical factors, ensuring treatment is provided to patients most likely to benefit. Infants meeting these criteria should be prioritised in the selection process, rather than selection being based on SMA type alone.

International practices offer sound recommendations on the types of criteria to be considered. In Europe, treatment using OA was restricted to infants under two years or weighing less than 13.5 kg due to reduced efficacy beyond these thresholds (Kyriakides, 2021). Taiwan's approach of prioritising treatment according to infants' motor function scores post-diagnosis offers another useful benchmark (National Health Insurance

Administration, 2024), with patients with higher scores more eligible. Patients with higher scores retain more measurable motor function, making improvements easier to detect. Additional criteria to consider include excluding patients with sustained or invasive ventilation requirements, as seen in Brazil (Guimarães, 2023).

While these international practices offer a framework, recommendations that are tailored to the local health and socioeconomic context must be prioritised. Malaysia is a middle-income country with a health system that relies on a combination of tax-based government services and a growing private sector. However, Malaysia's health budget is limited, and the allocation for treating rare diseases is similarly constrained. Compared to countries like Taiwan and Italy with their comprehensive national healthcare, Malaysia must consider its more limited coverage, budget constraints, and developing healthcare infrastructure when formulating eligibility criteria.

SMA most frequently manifests in early infancy, with the more severe forms typically presenting before six months of age. Evidence consistently shows that all disease-modifying therapies, particularly gene therapy, achieve the greatest clinical benefit when administered as early as possible, ideally before symptom onset. Malaysia's public health system, though comprehensive, still faces slow detection rates and limited genetic testing capacity, with parents reporting waiting up to four months for a confirmed diagnosis. Recognising the challenges of early diagnosis in Malaysia's healthcare system, setting a criteria that is tailored to the local diagnostic capacity and resource constraints ensures long-term sustainability and cost-effectiveness. These challenges underscore the need to align eligibility criteria with Malaysia's diagnostic readiness and early intervention capacity.

Early detection through newborn screening (NBS) emerged as a recurring theme among both parents and clinicians. Interviews with parents of SMA patients presented a positive case for its implementation; they saw early intervention in the health system as supporting better informed choices. In the context of the Malaysian healthcare system, the implementation of NBS faces several challenges, including logistical difficulties in rural regions, limited nurse training, resistance to programme adoption, and a lack of sustained commitment and funding from the relevant ministries. Despite this, the potential of NBS remains substantial; a cost-effective option would be to screen for multiple conditions simultaneously, and to be risk-based in nature rather than targeting individual diseases in isolation. An expanded NBS programme would provide both early diagnosis and prevent a lifetime of impairment for many rare disease patients (Thong et al., 2019). Notably, the Institute for Medical Research (IMR) is in the process of conducting a performance test for its own newborn screening test kit for SMA, through a pilot study assessing population coverage. The initiative is a positive step towards the broader implementation of rare disease screening, but requires sustained funding and the development of clear national policies.

In terms of procurement for high-cost treatments, eligibility criteria serve as a tool for forecasting strategic demand as well as cost containment in upper-middle income countries. By setting clear definitions of who qualifies for the treatment, the health systems in place, in this case Malaysia's PASc, can more accurately estimate the required future

costs and ensure efficient allocation of resources, managing the financial pressures while still allowing patients access to care. The criteria would also allow for more standardised access to available treatments.

An ideal criteria development process would be coordinated by the Ministry of Health and developed inclusively with input from various stakeholders. The Ministry would then be better equipped to engage in contract negotiations with pharmaceutical companies and to undertake necessary health system uplift to support treatment roll out. The Malaysian Health Technology Assessment Section (MaHTAS) could work under MOH's purview to provide evidence-based input on the criteria. In lieu of such a process, IDEAS has undertaken this research to develop and suggest criteria, and did so with the support of an inclusive pool of stakeholders.

IDEAS consulted widely to gain stakeholder insights on possible eligibility criteria for SMA gene therapies. This included a focus group discussion held with clinical professionals that provided several clinical recommendations. Of those present, many had experience treating the different types of SMA in both the public and private sectors and were familiar with gene therapies, with expertise in paediatrics, neurology, genetics, pharmaceuticals, and medical research.

In order to be clinically appropriate and cost-effective, OA should be administered to patients with a confirmed diagnosis of SMA and no more than three copies of the survival motor neural 2 (SMN2) gene. This threshold helps to ensure the treatment goes to patients most likely to experience clinical benefits and for whom the therapy offers the greatest value within a resource-limited healthcare system. Eligible patients should also be under 12 months old at the time of screening, pre-symptomatic, and without life-threatening symptoms. Patients with no more than three copies of the gene experience rapid motor neuron loss in infancy, and OA works best when administered early, making the under 12 month timeline and presymptomatic the most effective with highest likelihood of patients having measurable benefits. Clinicians agreed that they would need to adapt these based on the local healthcare infrastructure, as well as the realities of local diagnostic capacity that is still lacking for rare diseases, and specifically in the context of SMA that is harder to diagnose at early stages.

While there are clear benefits to having predefined eligibility criteria, equitable access remains a point of contention among those living with SMA. About one-third of interviewed parents opposed the idea of limiting treatment access to only pre-symptomatic or younger patients. Parents expressed concern that limiting who gets access to life-saving medications by the virtue of pre-set conditions would reduce hope in a community already struggling with scarce resources, suggesting instead that patients that miss a few of the criteria can still be considered during the selection process, especially given the often months-long process of diagnosis that may make some patients ineligible. *"Give equal opportunities to all SMA patients. It doesn't matter whether the person has hope or not, let everyone have the chance to experience the treatment. Don't look at their family background or anything like that" (S08)*. Such sentiments reflect broader anxieties within

the SMA community about being excluded from potentially life-changing treatments due to rigid age or functional cut-offs.

Malaysia's eligibility criteria for SMA and similar high-cost treatments must balance scientific rigour with social justice, while remaining adaptable to both current and future diagnostic capacities. The clinical criteria as suggested by clinicians, including a confirmed diagnosis, no more than three copies of SMN2 gene, and being under 12 months of age are suggested as a framework for targeting patients most likely to benefit from treatment. With more comprehensive NBS, stricter criteria could be applied, prioritising pre-symptomatic infants and those with early detectable motor function deficits to maximize clinical outcomes and resource efficiency. In the current context, where NBS coverage is limited and early detection remains challenging, slightly more flexible criteria may be needed to avoid excluding eligible patients who experience diagnostic delays.

Importantly, these criteria can define publicly funded access, while families who can afford treatment privately may still pursue therapy beyond these boundaries. This approach ensures efficient allocation of limited public resources without denying opportunities to patients whose families have the means to pay, acknowledging both financial realities and patient perspectives. Consultations with caregivers highlighted the emotional importance of hope and inclusivity, emphasizing that rigid cut-offs may cause distress in a community already facing scarce resources.

While prioritising patients most likely to benefit ensures efficiency and sustainability, policies must also acknowledge the emotional and ethical imperatives voiced by caregivers. Establishing a transparent, inclusive, and evidence-informed eligibility framework anchored in newborn screening, clinical data, and patient equity would represent a critical step towards operationalising equitable access to gene therapy under Malaysia's evolving health financing ecosystem.

4.2 Outcome Milestones

Payments in stages based on treatment response ensures accountability from the providers, as OBPMs set payments against outcome milestones that are clearly defined and measured at regular intervals. The risk-sharing nature of OBPMs links revenue directly to the performance of the therapy offered, and forces pharmaceutical companies to stay transparent in data collection, as a way of proving and tracking the efficacy outside of trials. The risk-sharing approach also ensures that these companies are incentivised to better support the relevant stakeholders in identifying patients that would benefit the most, prioritising equitable access rather than profit.

In the case of OA, from a clinical perspective, a five-year period of key check-ins is recommended to ensure both clinical effectiveness and accountability. Outcomes evaluations are commonly scheduled at 6 months, 12 months, 24 months, 36 months, 48 months, and 60 months post-administration of the treatment (Bitetti et al., 2023). The check-ins should assess a combination of clinical outcomes, namely motor function

improvement, survival without ventilator support, and developmental milestones. The CHOP INTEND score[2] is a recommended reliable indicator, with higher scores reflecting the treatment's overall effectiveness and long term benefit.

Similar to eligibility criteria, outcome milestones must be tailored to the local context of health and socioeconomics. International practices for predefined clinical outcomes include motor function [Brazil] (Guimarães, 2023), survival without permanent ventilation [Italy and United Kingdom] (AIFA, 2021), and event-free survival (Portugal's proposed model) (DeFigueiredo et al., 2022). These examples demonstrate how reimbursement and outcome milestones are quantified in high-income countries, and as such cannot be directly applied to the Malaysian healthcare system due to a lack of monitoring infrastructures in the form of registries, as well as reliance on a mixed public-private healthcare financing, unlike the United Kingdom, Italy, and Brazil's high government healthcare expenditure. This recognises that Malaysia lacks the health budget to match these systems in the short-term, but does not excuse it from aspiring to reach similar expenditure levels in the future.

Malaysia's healthcare financing shares some common ground with other ASEAN-6 nations, such as the presence of a mixed public-private system in some countries and government involvement in funding. However, the specific mechanisms, the degree of reliance on government funding versus out-of-pocket (OOP) expenditure, and the presence and nature of social health insurance schemes vary considerably, emphasising that Malaysia must develop its own context-specific framework rather than adopting from other countries in the region (Gill et al., 2024).

In addition to clinical indicators, social indicators such as quality-of-life, social participation, caregiver satisfaction, out-of-pocket expenditures and healthcare dependency (hospitalisations and medical emergencies) should also be a significant factor taken into account (López-Bastida et al., 2017) when looking at outcomes. The Ministry of Health or respective governing body of the OBPM should work with various stakeholders to identify a set of clear outcome milestones, based on clinical recommendations and patient input.

An independent committee of specialists should be formed to oversee and assess outcomes. It should consist of the appropriate specialists in relation to SMA care, including but not limited to a paediatric neurologist, paediatric developmental specialist, paediatric pulmonologist, clinical geneticist, speech therapist, and physiotherapist for a clinical perspective, as well as representatives from the financing and procurement divisions among others. An independent board would ensure objectivity and eliminate any potential bias that may arise from assessments conducted solely by the patient's primary clinician. It is necessary to establish an independent clinical committee to review the milestones.

[2] The Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND) examination is a tool designed to help measure motor abilities in children with SMA, originally designed as a test for infants presenting with SMA type 1.

Akin to the eligibility criteria, an MOH-led process for developing outcomes in consultation with system stakeholders would be ideal. In lieu of that, this paper engaged a wide range of clinicians, patients and expert others to develop outcomes measures that take into account social as well as clinical factors.

For both administrative simplicity and significance reasons, clinicians recommended survival as a main outcome linked to payments, with a five-year survival period deemed appropriate. They also emphasised that the presence of complications during treatment (e.g. Scoliosis, joint issues, feeding support, ventilator support, pneumonia-free) be monitored, as their presence and other health complications would convey reduced efficacy of the treatment and disease progression. The overall social impact on patients and caregivers alike was also stressed as an important marker for success, with a higher or increased QOL recorded through surveys or interviews considered a successful outcome. Clinicians also agreed that the price charged for the overall treatment should be lowered if these complications do arise during the set check-in periods. Their inputs highlighted a need to balance clinical effectiveness with social relevance, providing a foundation for MOH to adapt when implementing OBPM in Malaysia's rare disease framework.

Interviews with key informants revealed significant concerns of the outcomes-based model itself. A majority (60%) expressed concerns, primarily around the fairness of applying uniform milestones to a highly variable disease. Others questioned the durability of the therapy and the power dynamics involved, noting that the payer has the power to decide which outcomes matter (S06), potentially creating conflict between clinical, financial, and patient priorities.

A critical ethical concern in implementing OBPMs for SMA gene therapy is the risk of creating perverse incentives that undermine fairness and clinical integrity. If reimbursement is tied too rigidly to predefined clinical milestones, providers may feel pressured — directly or indirectly — to preferentially select patients who are most likely to meet outcome targets, thereby excluding those with more severe disease presentations. Such structures may also incentivise clinicians to manipulate, selectively report, or over-interpret outcomes to avoid financial penalties, compromising both ethical practice and data quality. Families caring for children with complex or advanced SMA may be disproportionately penalised if the model does not account for inherent variability in disease progression. Further, FGDs also highlighted the importance of adjusting payment structures to avoid discriminating against severe cases, with suggestions to apply higher discounts (e.g. reducing baseline cost by 25–50%) for children with poorer prognostic indicators. This aligns with the broader consensus from KIIs, where 60% of participants disagreed with outcome-linked payments, noting that KPI milestones are often unfair given the heterogeneity in disease progression, variable timing of therapeutic response, and unrealistic expectations of cure. One parent expressed this sentiment succinctly: "You can't base things on outcomes... For example, you say 20% motor skill improvement. But... others, it's really hard. So... to achieve this skill for SMA it's unfair" (S04). Half of the participants further acknowledged that for many families, stabilisation — preventing deterioration rather than achieving improvement — constitutes a meaningful success.

Quality of life after treatment is of great importance to those affected by SMA. Rather than quantifiable clinical outcomes, 40% of key informants preferred the efficiency of medication to be measured through an increased quality of life that encompassed both psychosocial and physical factors. They viewed successful treatment as translating into a better social life, ability to move around independently and better autonomy, allowing SMA patients a better quality of life overall. A stark number (50%) of parents reported being denied access to public education due to their children's SMA diagnosis, often hindering their social life and participation in activities outside of their homes. A significant amount (50%) of key informants acknowledged that while recovery is almost impossible due to the nature of the disease, stopping progressive symptoms would *"actually be considered a kind of success for them." (S08)*

These local perspectives mirror concerns raised in international evidence regarding OBPMs for high-cost therapies. Studies have warned that tying payment to narrow clinical endpoints risks disadvantageous patients with poorer baseline function and can unintentionally promote patient selection bias. Researchers have also highlighted that gene therapy outcomes are highly variable and may take months or years to manifest, making short-term KPIs ethically problematic and potentially misaligned with real-world patient trajectories (Michelsen et al., 2020). Collectively, evidence from both Malaysian stakeholders and global scholarship underscores the need for a carefully designed financing model — one that avoids penalising severity, protects families from undue burden, and recognises stabilisation as a legitimate therapeutic success in SMA.

In conclusion, Malaysia's outcome milestones for SMA and similar high-cost treatments must take both clinical efficacy and social wellbeing into account, as well as the feasibility of monitoring said outcomes in the health system. The main recommendations put-forth are payments linked to a five-year survival period, the absence of further health complications during treatment, and increased QOL measures. These suggestions would help ensure that future efforts to adapt an outcomes-based system are reflected in a transparent, equitable and sustainable framework.

4.3 Health Systems and Governance

Greater health system awareness of SMA and accessibility is needed to improve the patient experience. The lack of awareness on rare diseases, especially SMA in the present healthcare system has a direct impact on care. Parents report a lack of infrastructure, as well as vastly different experiences depending on the hospital (70%), with one family noting that a (public) hospital they visited previously did not have any support for their child, while expressing gratitude for a different, more experienced hospital where the doctors have helped a lot. This disparity necessitates the urgent need for standardised protocols and training across the healthcare system.

Establishing a national registry for rare diseases is essential for advancing healthcare delivery. A registry allows access to detailed patient data and supports a more efficient and effective use of health system resources. With a set of guiding eligibility criteria in

place, a national web-based platform would support optimal care tailored to the patient and provide a better understanding of the disease burden. As an interim measure before a registry is fully developed by MOH under the National Rare Disease Policy, clinicians recommend leveraging the existing under-five mortality rate (U5MR) data.

Financial constraints remain a significant barrier to reform, with rare diseases often limited by a tight annual budget cycle. A crucial recommendation from key informants was to increase government financial support for rare diseases in the way of subsidies and national funds. It is also important to acknowledge that rare diseases naturally get deprioritised in national budgets due to their high per person treatment costs. Many of the key informant interviewees (60%) saw the low rate of healthcare spending as a share of gross domestic product as a critical barrier to accessing treatments and better care. Patients and families instead pay high out-of-pocket for medication and hospital visits, with fees often amounting up to RM100,000 per year. These constraints need to be recognised while still advocating for innovative financial solutions that may help stretch limited resources.

4.4 Procurement

As a critical cog in the treatment access machine, the procurement discussion is central to exploring OBPM and whether it can be operationalised in Malaysia. Currently, innovative or rare disease treatments are largely channelled through the MASc framework, which includes PASc and other compassionate use mechanisms. This framework does include mention of performance-based schemes. But due to there not having been uptake, payment schemes under these frameworks remain financial-based rather than performance-based, with payment tied to treatment supply rather than verified outcomes.

Targeted reform and improvements must be made to the procurement system to better support access to high-cost therapies. Administrative complexity often results in delays in the procurement process, usually taking six to nine months for the tender to be awarded (Thanimalai et al., 2022). The system must be upgraded to better support multi-year contracts as would be required by OBPM. The procurement division of MOH had noted that integration of OBPM would require modifications to existing procurement workflows, as the process of procurement is layered and goes through several stages and ministries before a framework can be built. Navigating the sheer number of government areas involved can represent an overwhelming barrier to promoting an innovative approach.

Interviews with various stakeholders highlighted a shared recognition that implementing OBPMs requires multi-level governance. Procurement officials noted that while MOH can initiate contractual and technical frameworks, fiscal oversight (for example) and risk-sharing arrangements would necessitate coordination with the Ministry of Finance.

OBPMs serve to make high-cost therapies more feasible within limited health budgets. Splitting payments out over several years creates more space within the budget to provide equitable access. At the current moment, there is only RM25 million set aside from the

healthcare budget for rare diseases (Abu Bakar, 2025), with this figure tied to pre-existing programmes of medications already approved and in use. Various stakeholders also noted that pooled purchasing in the ASEAN region could be a key step in reducing fragmentation and improving bargaining power with pharmaceutical companies, especially in the case of high-cost therapies.

Another barrier to procuring emerging therapies is timely access to information and expertise by relevant health officials. Focus group participants raised the need for civil society organisations, academic experts and clinicians to support timely capacity building of officials on emerging diseases and treatments. There is space for greater advocacy, supported by further investment in research, to build capacity in Putrajaya to understand emerging treatment options sooner.

Discussions reaffirmed that the main barriers to OBPM adoption include a limited yearly budget, lack of pre-existing framework or model, knowledge deficits, and long bureaucratic processes standing in the way. However, enabling factors were also identified, such as the recent launch of the National Policy on Rare Diseases (2025), growing political appetite for innovative financing, and existing collaborative mechanisms under MASc that could serve as entry points.

4.5 Financing and Health Economics

A payment stream for prohibitively expensive, one-off therapies is needed. The prospects for Malaysians with SMA to access expensive gene therapy treatments are very poor under the prevailing payment model. The one-off treatment cost, of almost RM9 million on top of the supplementary, ongoing costs of patient care, is far beyond the reach of nearly all households in Malaysia. While no precise data exists on how many Malaysians could afford such treatment, available national statistics suggest it would be a tiny fraction of the population. Even among the T20 (top 20% income group), the highest mean monthly household expenditure in 2016 was only RM17,292 (or RM207,504 annually), according to the Department of Statistics Malaysia (2020). The cost of a single gene therapy treatment course is more than 40 times this.

The cost of gene therapy is also very large from a public finance perspective. Malaysia's per capita health expenditure in 2023 was RM2,521 (Malaysia National Health Accounts [MNHA], 2024b), so spending upwards of RM9 million for gene therapy and associated long-term care for one patient presents a large and difficult decision for health and budget officials. It equates to roughly 3,730 times the annual per capita spend.

Despite positive developments towards deepening support for rare diseases, there has been no indication that gene therapy for SMA will be subsidised through government procurement in the near future. Without a sustainable payment mechanism, access will remain limited to only the richest Malaysian households, likely well below 0.001% of all households.

There was near universal support among stakeholders for a more manageable payment stream to improve equitable and affordable access. Clinicians and patients in particular are attracted to financial models that remove the barrier of steep one-off costs, and would be willing to incur the administrative time costs associated with verifying outcomes should they be linked to payments. Payment streams do not resolve access barriers for most households, but spreading the financial impost over several years could make government procurement and financial pooling more palatable.

Determining the duration of the payment stream and the number and size of payments is a matter for negotiation between the treatment provider and MOH. There appear to be few impediments to developing a pure financial payment stream under existing procurement processes, with several successful examples in recent history. Given the large difference between annual per capita health costs and the size of one-off treatment, streams would need to be spread over a significant time horizon to make it economic. A period of 15-20 years would reduce annual per patient payments to around RM400,000–RM600,000, comparable to the expenditure on a high-cost hospital case.

Assuming there are around 30 to 50 new SMA cases annually, with SMA type 1 accounting for around 60% of cases (Verhaart et al., 2017), the total upfront cost in the absence of a payment stream would range between RM270 million and RM450 million per year. This would amount to roughly 0.3–0.6% of total national health expenditure, or 0.7–1.1% of public health spending. However, if the cost were spread across 15–20 years through an outcome-linked payment stream, the government's annual fiscal commitment would fall to about RM13–30 million in the first year and not reach RM270 to RM450 million per year until a full 15-20 year cycle has occurred. Such a structure would significantly reduce the short-term present value of funding this treatment.

Payment streams can theoretically reduce the sticker price of therapies in real terms, subject to the treatment provider's terms. Simply put, if the treatment provider is willing to spread payments over a long period without charging interest or adjusting for inflation, the cost of the therapy reduces in today's money. An implicit discount such as this — alone or accompanied by a further sticker price discount (for example, to account for Malaysia's lower capacity to pay as a middle-income country) — could encourage both government procurement and financial pooling options.

One important caveat to this approach is that the government would be wise not to lock itself into such an agreement for the full payment lifecycle. Treatment costs could reduce sharply should competitor treatments become available, so MOH should ensure it does not make long-term commitments guaranteeing future cohorts. It may be practical, for example, to renegotiate the payment stream for new cohorts every two to three years. And where the payment stream extends beyond the life of the intellectual property protection, to use the potential for future generic competitors as leverage to reduce the overall payment price.

4.5.1 When should a payment stream be linked to outcomes?

This research specifically explored whether a payment stream for SMA patient access to gene therapy should be outcomes-based. In essence it sought to answer the counterfactual question: in what circumstances would linking a payment stream to clinical eligibility and outcomes support access compared to simply spreading out payments?

Precedent in other countries suggests outcomes-based payment approaches work best when treatment outcomes are relatively uncertain. They were first developed as a way to balance access and accountability for emerging therapies that remain experimental or lack long-term data. This encourages countries to acquire therapies that could help patients while mitigating the political and financial risks of outcomes being worse than hoped.

For example, Italy reimburses Zolgensma under a 'payment at result' agreement, with performance checkpoints at 12, 24, 36, and 48 months, and partial refunds if agreed milestones are not met (Eversana, 2021). Similarly, Brazil adopted a five-year risk-sharing agreement with Novartis, under which payments are made in annual instalments and refunded if developmental milestones are not achieved (Guimarães, 2023). Portugal has also proposed a performance-linked reimbursement model combined with annuity-style payments over 5–15 years to reduce upfront budget pressure (De Figueiredo et al., 2022). These arrangements, however, come with substantial administrative complexity and depend heavily on robust clinical data systems to verify patient outcomes. They also occurred at a time when treatment outcomes were less certain.

Procurement and health experts reinforced the argument that outcomes-based payments are best reserved for uncertain treatment prospects. They noted that there is little rationale for linking payments to outcomes if clinical prospects are predictable, as the model reverts to a payment stream with an additional administrative burden linking payments to outcomes. As one insurance representative explained, outcomes-based payments are good for addressing uncertainty when you are not sure if the drug really works. Put simply, if outcomes are knowable, a pure payments stream is more administratively and cost effective.

Indeed, in the case of SMA and gene therapy, treatment outcomes are now well-characterised and fairly predictable, though it should be noted that the evidence is still evolving for outcomes beyond a decade's timeframe. Multiple real-world and clinical studies demonstrate substantial motor-function improvement and survival benefit, especially when administered early (Ogbonmide et al., 2023; Ruggiero et al., 2024; Zhang et al., 2025), with efficacy recorded for a 7.5 year period post-treatment (Gowda et al., 2024). Under such circumstances, there is limited rationale for linking payments to outcomes as the additional monitoring and verification processes would add cost and administrative burden without meaningfully improving value for money.

However, this does not invalidate the broader policy relevance of OBPMs. OBPMs could and should be explored for other emerging therapies where uncertainty is genuine.

For example, treatments still in early evidence stages, gene therapies for less-studied rare diseases, or first-in-class interventions with limited longitudinal data. In these contexts, outcomes-linked arrangements can protect the procurers against unforeseen underperformance while encouraging innovation and access.

As discussed earlier, from a procurement standpoint, operationalising OBPMs in Malaysia would require several foundational steps. First, a national treatment registry or outcomes database should be established to capture real-world performance data. Second, MOH would need to develop a standardised contract template that clearly defines refund clauses, outcome milestones, and mechanisms for dispute resolution. Finally, OBPMs should initially be piloted for high-uncertainty, low-volume therapies to build institutional experience before broader implementation. By distinguishing between therapies with predictable and uncertain outcomes, Malaysia can better align its payment mechanisms with the level of clinical and financial risk.

Recommendations for a financial payment stream to make gene therapies accessible to SMA patients need not be linked to outcomes. A pure financial payment stream would be more efficient. However, for other rare diseases and emerging therapies where long-term effectiveness remains uncertain, OBPMs should be explored as a complementary approach to manage clinical and financial risk. This would allow Malaysia to build the necessary data infrastructure and procurement experience to support evidence-informed access to future high-cost, high-uncertainty treatments.

4.5.2 Determining the expected financial risk and cost burden of rare disease treatment

A fundamental building block to securing funding to treat rare diseases including SMA — whether emerging treatments like gene therapy or longer standing options — is to establish the financial profile of the disease and treatment. Regardless of who funds the treatment (households, government, insurers) and via what mechanism (individual or pooled, upfront or via a payment stream), understanding the expected costs is an essential prerequisite.

The financial profile of rare disease treatments and emerging therapies is not systematically produced in Malaysia, creating a critical information gap. When a new treatment is approved for use, there is no requirement for the provider or a ministry to determine the estimated number of patients with the disease, to make commitments regarding or list the sales price for the treatment across the treatment lifecycle, nor to detail the associated costs of the patient's treatment (clinical fees, other medicines, physical and psychological therapy etc.). Emerging treatments like gene therapy for SMA patients are granted access to Malaysia without regard to establishing a market or a supporting clinical ecosystem.

There are sound public policy and public good arguments for establishing a centralised process to alleviate the information gap. The Ministry of Health assesses clinical evidence before approving access to treatments in the interests of patient safety, but by not

simultaneously considering how patients can affordably utilise these treatments is not attending to equitability concerns. Expensive treatments are made available to high-wealth households, whereas ordinary Malaysians resort to crowdfunding or are frustrated at missing out on potentially superior clinical options. Systematising the assessment of critical financial information — alongside establishing eligibility criteria and supporting health ecosystem adjustments — would help capture the social value associated with facilitating equitable access to treatment, which is greater than the sum of the value to individual stakeholders if left to assess this themselves.

For new treatments, the ideal time for financial specifications to be determined is at the point of entry. That is, MOH could require this information from treatment providers before approving the therapy for use in Malaysia. However, it may not be necessary to mandate the provision of this information; treatment providers have incentives to volunteer it should it help facilitate reciprocal efforts to fill information gaps.

For existing therapies for which affordable and equitable access remains to be established — like gene therapy for SMA patients — treatment providers can opt into an equivalent assessment process.

A practical, centralised, scalable, impartial, and affordable implementation approach is recommended. It is important that the process avoid delays to therapy access and not impose an undue compliance burden on applicants in terms of financial and time costs, recognising that health economic assessments can be prone to excess.

The suggested approach would be to strengthen an existing part of the MOH portfolio, potentially the Malaysian Health Technology Assessment Section (MaHTAS), to undertake such assessments. The assessment requires access to aggregate patient and health system information that may only be available to the government, and the government has access to and oversight of many financial and procurement levers. The chosen area should draw on expertise from multiple areas of government, including within the Ministry of Health and Ministry of Finance. Clear, transparent, and robust governance for the agency and process should be established, and outcomes of the assessment process should be made publicly available to maximise stakeholder buy-in. The expanded function could be funded on a cost recovery basis, with treatment providers paying a nominal fee to help develop market information at the point of therapy approval.

4.5.3 Leveraging economies of scale for equitable and affordable access

Emerging rare disease treatments like gene therapy are financially burdensome, creating a barrier to equitable and affordable access. This paper explores innovative payment options because financing for gene therapy has not developed organically since treatment became available, limiting patient access. As previously noted, one-off and novel treatments such as gene therapy create acute financial challenges with high upfront costs that are well beyond the means of Malaysian households and are difficult for policymakers to justify vis-a-vis other procurement priorities.

Financing for emerging SMA treatments and rare disease therapies more broadly could better leverage economies of scale. A financial burden of over RM9 million per patient — whether mostly paid upfront or spread over many years — far exceeds most household budgets and remains a significant outlay for governments with finite resources and many competing priorities. And it remains a significant outlay for governments regardless of whether the money comes from consolidated revenue (the Budget) or a dedicated fund. But the economies of scale look more favourable when they are spread across a larger funding pool, like insurance policyholders.

Treatment costs are relatively modest if effectively spread across a larger population, but Malaysia lacks mature pooling mechanisms. Underdeveloped insurance markets are a major challenge for funding treatments for SMA, rare diseases, and any treatment in Malaysia. Malaysia does not have a national insurance fund and private insurance contributes only 8.3% of total health expenditure (MNHA, 2024a) (see Figure 1). Only around half of all Malaysians have health insurance, which reduces both the population contributing to the pool and the number of people covered (Yunus, 2024). Utilising insurance to spread rare disease treatment costs across a large population pool should be a medium-term objective, but it requires further insurance market deepening.

Figure 1: Malaysia's total expenditure on health by sources of financing, 2022 (MNHA, 2024a)

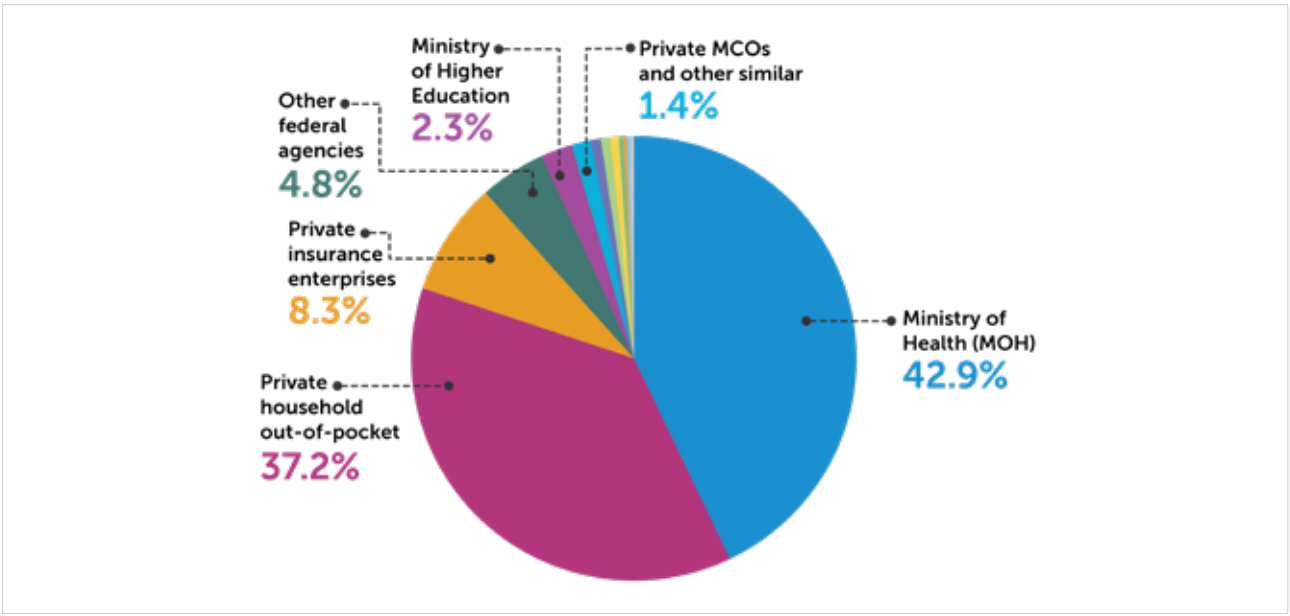
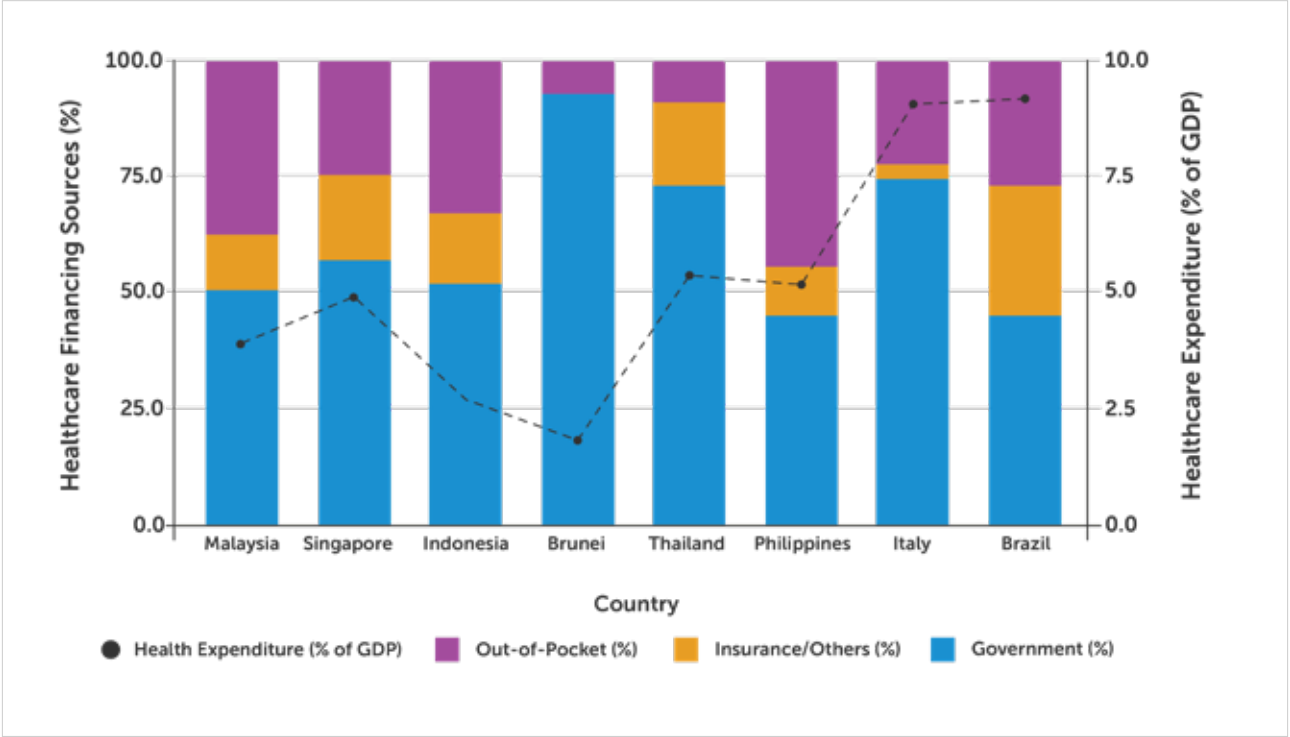


Figure 2 illustrates the percentage of healthcare financing sources in comparable ASEAN member states, highlighting how Malaysia's reliance on public and private spending compares to its peers regionally. Italy and Brazil serve as examples of countries with high government spending in healthcare expenditure, with existing programmes to support high-cost gene therapies, providing a benchmark of what strong public financing of healthcare looks like. The black line illustrates the percentage of healthcare expenditure of gross domestic product (GDP).

Figure 2: 2022 Healthcare Spending (% of GDP) by Country, 2022
(WHO Global Health Expenditure Data and World Bank (current health expenditure as % of GDP)



To understand the potential economies of scale from pooling, Figure 3 illustrates a RM9 million treatment impost as a share of different budgets, including a scenario in which Malaysia hypothetically develops a mature insurance system comparable to Australia's. It also illustrates the potential benefits of spreading payments over time under a stream such as that considered under an OBPM.

Table 2: Comparative analysis of RM9 million treatment burden

Financial Benchmark Category	Annual Amount (RM)	RM9 Million Burden as a Percentage of Annual Amount (%)
1. Median Household Disposable Income	71,988	12,501.90
2. Average Annual Health Spending (Per Capita)	2,521	356,956.50
3. Total General Insurance Market	21,400,000,000 (RM21.4 billion)	0.04
4. Hypothetical Mature Insurance Pool (Australian benchmark: RM2,386 premium × 15.39M contributors)	36,708,000,000 (RM36.71 billion)	0.03

Notes:

1. *On median household disposable income: Derived from the Household Income and Expenditure Survey (HIES) 2024 Report (Rahim, 2025)*

2. *On average annual health spending (per capita): From Malaysia National Health Accounts Steering Committee Meeting presentation (2024)*

3. *On insurance market size: The total general insurance market size uses the General Insurance Association of Malaysia's (PIAM) reported gross written premiums (GWP) of RM21.4 billion in 2023 (PIAM, 2024).*

4. *On mature insurance pool calculation: Applying Australia's average premium as 3.3% of household income (HMD Insurance, n.d.) to Malaysia's median household income (RM71,988) gives an aspirational annual premium of approximately RM2,386 per worker, which across 15.39 million workers would generate a mature insurance pool of about RM36.7 billion per year.*

The sizable differences demonstrate that financing rare disease treatments requires large-scale financial pooling mechanisms to leverage economies of scale. Furthermore, exploring the potential benefits of spreading payments over time, such as under an OBPM reveals its relative impact on the per annum costs for different entities, as illustrated in Table 3.

Table 3: Impact of Spreading Payments Over Time

Financial Benchmark Category	1 - Year Burden (%)	5 - Year Burden (%)	10 - Year Burden (%)	15 - Year Burden (%)	20 - Year Burden (%)
1. Median Household Disposable Income (RM71,988/yr)	12,501.90	2,500.40	1,250.20	833.50	625.10
2. Average Annual Health Spending (Per Capita) (RM2,521/yr)	356,956.50	71,391.30	35,695.65	23,797.10	17,847.80
3. Hypothetical Mature Insurance Pool (RM36.7B/yr)	0.025	0.005	0.003	0.002	0.001
4. Hypothetical Annual Insurance Pool (RM7.695B/yr)	0.12	0.02	0.01	0.01	0.01

This table shows that while extending the payment over time significantly eases the annual strain for a household (e.g., from almost 12,502% to 625%), the burden remains overwhelmingly unaffordable. For a large financial pool (such as an insurance fund), the impact is negligible, making such financing mechanisms the only viable option. The hypothetical pool demonstrates how expanding the contributor base drastically lowers the per-person cost, even if only a few patients receive the RM9 million treatment in a year.

4.5.4 Identifying Favourable Characteristics for Pooling

Using gene therapy for SMA patients as an example helps identify the characteristics of rare diseases that offer the potential to utilise more favourable economies of scale. While the direct and associated costs of gene therapy are large on a per patient basis, the number of patients for which gene therapy would be clinically determined to be medically optimal is very small in population terms. It is not only small but a relatively stable and predictable population, which is beneficial from a financing perspective as the expected risks and costs can be estimated with a high degree of accuracy. Subject to overcoming the information gap, whereby the risks and cost are identified (consistent with the recommendation of the preceding section), gene therapy for SMA patients has a favourable financial profile from the perspective of risk products like insurance.

An obvious challenge is that the exact number of patients, and therefore the cost to be pooled, is presently unknown. The incident rate of SMA in Malaysia is not presently known due to limitations in screening and data handling. It is believed to be around 1 in 10,000

persons, but this is based on overseas evidence. While this establishes a high starting point — as one interviewee pointed out, RM10 million ringgit per 20,000 (the oft-referred to but unverified rate in Malaysia) is RM500 per person, which would represent a significant increase in annual premium insurance costs for a single disease — not all patients would be recommended for gene therapy, therapy costs reduce over time, and financial pools earn investment returns that help meet costs.

What this unrealistically high starting point illustrates however, is the accompanying necessity of payment streams for emerging therapies with high upfront costs. This has been discussed earlier.

4.6 A Replicable Model for Rare Diseases

While not all rare disease treatments share gene therapy's financial profile, most share its characteristics. Rare diseases by definition affect small populations and they do not transmit in large and unpredictable patterns like infectious diseases. Subject to filling the equivalent information gaps, any financial pooling model developed for SMA gene therapy could be replicated for other (potentially all) rare diseases.

Leveraging economies of scale requires a two-step process to first deepen the insurance market and then ensure financial information gaps are filled for a larger number of rare diseases. Gene therapy for SMA can serve as a proof-of-concept case for a wider model benefiting patients with other rare diseases. The Ministry of Health and Ministry of Finance should seriously explore options to leverage economies of scale for rare gene therapy and other emerging treatments for rare diseases.

4.6.1 Regulating inclusive insurance coverage

As the previous section discusses, emerging treatments for rare diseases viewed through a household or government budget lens face steep financial positioning challenges. Most households can never afford expensive treatments, while governments struggle to justify large per patient expenses for few beneficiaries.

Insurance coverage represents an attractive potential alternative that utilises economies of scale to more favourably reframe the financial impost and decision. Spreading the treatment cost over Malaysia's workforce (formally around 16.9 million employed persons [DOSM, 2025]) or population 15 years and over (about 26.5 million [DOSM, 2025]) would reduce towards negligible the change to insurance premiums and investment earnings for gene therapy for SMA — and represent a potentially manageable cost for a broader set of rare diseases.

In addition to filling the above mentioned information gaps and deepening the insurance market, leveraging insurance to support rare disease treatment requires regulatory impetus. Insurers are slow and often unwilling to offer expanded coverage for emerging treatments, especially as requiring premium-sensitive consumers to opt in presents challenges to obtaining a sufficient insured pool to cover the expected risks.

One particular challenge for SMA and gene therapy is the presentation of the condition at young ages (as early as birth) coupled with treatment being most effective when delivered early. So insurance would need to be available from birth or those that would benefit most from treatment will not be covered.

Given these characteristics, Malaysian policymakers should consider [further] regulating default insurance coverage to improve inclusivity of treatments and diseases. As Bank Negara Malaysia (BNM), MOH and the Employees Provident Fund (EPF) contemplate the development of a basic health insurance product (The Edge, 2025), the inclusiveness of coverage must be balanced against cost. Increasing affordability for patients is not just achieved through generating greater pooling size and more efficient funds management, but by ensuring wider not low-frills coverage. Efforts to develop basic health insurance products should uplift not slash default coverage.

Specifically, BNM's requirements for medical insurance product design could be amended to require specified levels of inclusive coverage. BNM's Policy Document for Medical and Health Insurance/Takaful Business (2024) currently only requires providers to consider changing demographics and societal needs to ensure meaningful and inclusive product design (Bank Negara Malaysia, 2024). It does not compel providers to develop a default product with inclusive coverage, nor does it specify what inclusion is from a treatment and disease perspective. Requiring default coverage for rare diseases and emerging, approved treatments is the only path to their widespread inclusion in insurance contracts, as requiring Malaysians to opt in involves steep information barriers.

It must be emphasised that mandating default coverage cannot be done without first supporting insurers to understand the expected risks and costs of emerging treatments, and efforts to deepen insurance markets. Adding unspecified risks and costs without generating a supportive environment for industry growth will attract justifiable stakeholder opposition.

A full discussion of insurance market deepening is beyond the scope of this paper, but some aspects arising from stakeholder engagements are worth detailing.

Focus group and interview discussions emphasised a familiar recommendation that Malaysia needs a national medical insurance fund. Most mature health systems have compulsory or default public insurance pools that fund an extensive range of treatments. Insurance pools are funded by taxes or levies on individual or household incomes, with rates that usually increase with income. An intuitive approach to pursuing this in the Malaysian context would be to fund it through reallocated Employee Provident Fund (EPF) contributions. Malaysians are already able to withdraw a specified amount of their EPF (retirement) savings for health needs, but are limited by the amount in their accounts. In this sense EPF is a collection of millions of unpooled insurance funds that would be more effectively grown and more equitably drawn upon were they to be pooled. EPF contribution rates are well beyond what should be necessary to generate adequate retirement incomes, so diverting a share to a national insurance fund should benefit needy Malaysians now without compromising future living standards.

A related concern raised by financial sector stakeholders during interviews is how to maintain insurance policyholders over the long-term to help support pooled funding and payment streams. In the absence of tax or other policy incentives to maintain coverage, more active policyholders may adjust their coverage at certain milestones in a manner that runs counter to sustained pooling. For example, the characteristics of SMA and gene therapy may mean that it only makes financial sense to be insured for the first few years of life and drop the coverage thereafter. This would undermine efforts for insurers to spread treatments costs over a longer period whether payments are mapped to outcomes or not.

Insurance stakeholders also raised the need to develop reinsurance markets for policies that provide coverage for expensive rare disease treatments. A treatment cost of RM9 million per affected policyholder represents a substantial risk for small and less diversified insurers in particular. Reinsurance allows the risk to be spread across a larger insurance pool and be hedged against by the policy issuer. Industry players noted the absence of such reinsurance products and the deterrent this has on providing coverage.

Malaysia should set and work towards an objective of incorporating approved rare disease treatments as default inclusions in medical insurance policies. The deepening of insurance markets, potentially including the development of a national medical insurance fund, is a prerequisite for achieving this objective.

4.7 Ethics and Data Governance

4.7.1 Equity and Fair Access

Ensuring equitable and fair access to SMA gene therapy requires addressing the current structural and systemic disparities that disproportionately disadvantage patients from under-resourced centres and rural or remote regions. Findings from FGDs and KIIs consistently highlighted delays in diagnosis — often described as a prolonged "diagnostic odyssey" — stemming from limited specialist availability, inadequate clinical genetics services, and insufficient diagnostic infrastructure in many public hospitals. These inequities also extend to carrier screening for parents, high-risk pregnancy screening, and newborn screening, which remain largely inaccessible outside major urban centres. Approximately 70% of participants emphasised that low awareness among healthcare workers and the public significantly impedes early detection and timely referral, with severe geographical disparities noted in East Malaysia. Participants stressed that the current system tends to favour families who can access well-resourced centres, reinforcing unequal opportunities to receive life-saving therapies. The FGDs recommend establishing dedicated regional SMA centres in the north, south, east coast, and East Malaysia to decentralise expertise and strengthen local capacity. Furthermore, 30% of FGD participants advocated that all patients — regardless of socioeconomic background — should have equal opportunities to receive timely treatment and long-term support.

These local findings align with broader evidence, which positions patient access as a core issue of social justice and fairness in an era of high-cost gene therapies. Achieving

equitable access requires overcoming not only geographical and financial barriers, but also ensuring that all patients, regardless of socioeconomic status or location, can benefit from innovative, life-saving treatments. This challenge is amplified in gene therapy due to the prohibitively high prices, which place immense financial strain on healthcare budgets—particularly in lower-income countries or systems with constrained public resources. Such costs raise profound ethical and distributive concerns about sustainability, prioritisation, and the broader implications for healthcare resource allocation. Evidence from the European Union illustrates how high gene therapy prices have exacerbated disparities in access across Member States: While EU-level mechanisms aim to promote equity, wealthier countries remain better positioned to procure and sustain gene therapy programmes, widening the gap with lower-income nations (Reckelbus et al., 2025). This financial divide intensifies debates on distributive justice, including whether substantial public funds should be allocated to costly therapies for relatively small patient populations when these resources might otherwise address more prevalent health needs. Together, these insights reinforce the importance of embedding an equity-focused approach in Malaysia's SMA gene therapy strategy.

4.7.2 Transparency and Ethical Governance

Transparent and accountable governance is essential to ensure public trust and ethical implementation of SMA gene therapy, particularly within an outcome-based financing model. FGDs strongly recommended the establishment of an independent expert committee to assess outcomes, with mandatory representation from patient advocacy groups to ensure that lived experiences meaningfully inform decision-making. Approximately 30% of KII participants emphasised the importance of looking beyond clinical outcomes to include parental wellbeing, caregiver burden, and patient-reported experiences, cautioning that outcome measurement frameworks should not impose additional stress on families. An ethical governance structure must therefore clearly articulate how eligibility criteria are set, how outcomes are assessed, when payment triggers are activated, and how access to therapy is prioritised. This includes instituting transparent reporting mechanisms and ensuring that procurement and financing arrangements are openly communicated and acceptable to all stakeholders. Transparency is also necessary to clarify what can realistically be achieved with limited resources, preventing unrealistic expectations. Evidence from Europe highlights similar concerns, particularly in relation to differential pricing of gene therapies across countries. As noted by Elegido, price discrimination—where the same therapy is sold at different prices depending on buyer characteristics or geography—may be ethically defensible when it improves access for disadvantaged populations. However, in the European Union, these opaque pricing negotiations have also risked widening disparities between Member States and raised questions regarding procedural justice, including whether patients and payers can meaningfully evaluate or influence decisions that determine their access to high-cost therapies(Reckelbus et al., 2025). This underscores the importance of ensuring transparency, fairness, and participatory governance in Malaysia's approach to SMA gene therapy financing.

4.7.3 Supporting Patients and Caregivers While Ensuring Ethical Access

An ethically robust framework for SMA gene therapy must recognise and actively mitigate the financial, emotional, and social burdens that families shoulder throughout the diagnostic and treatment pathway. A core ethical principle is that access to life-saving therapy should never be compromised by a family's socioeconomic circumstances, nor should financing models exacerbate existing vulnerabilities. Insights from KIIs indicate that 60% of participants believe government support—through compassionate financing approaches, such as co-payment caps, tiered subsidies, interest-free payment schemes, EPF-linked mechanisms, or structured patient-access programmes—is essential to prevent catastrophic financial hardship. One caregiver explained the harsh financial reality: "At least have a plan... an installment scheme without interest, or EPF withdrawal... Parents can contribute part of the amount, like 10% or 30%, through installments. That would help a lot... Because in the end, we can't ever fully afford it." (S04) and noted even partial cost-sharing would be impossible without structured national support.

Beyond financial strain, caregivers face profound emotional and psychosocial challenges. Many (60%) reported life disruptions such as early retirement, job loss, sleep disturbance, restricted mobility, and fear of returning to hometowns with limited services. Children with SMA often face barriers in schooling, with 50% experiencing rejection or inadequate support due to disability-related misconceptions, requiring parents to seek advocacy from NGOs or escalate cases to district and state education offices to secure basic educational rights. Psychological distress is widespread; 20% of caregivers described feelings of hopelessness, grief, and acute stress, with one parent noting that the entire family urgently needed psychological support upon receiving the diagnosis (S04). Patient advocacy groups play an indispensable role in filling systemic gaps — 80% of caregivers cited NGOs as essential sources of emotional support, financial relief, information, community-building activities, and policy advocacy, including direct representation in Parliament. However, reliance on crowdfunding remains unsustainable and inequitable, with 50% reporting failed campaigns, online harassment, or inadequate funds despite urgent need.

4.7.4 Data Governance

Robust data governance is essential to ensure that SMA gene therapy is implemented in a manner that is clinically meaningful, culturally sensitive, and ethically justified. Clear rules on data ownership and stewardship are needed to protect patient rights while enabling responsible use of outcome data for national planning. Patient-level clinical and performance data should remain under national stewardship, with ownership residing collectively with the healthcare system and the individuals who contribute the data, rather than with industry partners. Any manufacturer access must be tightly regulated, purpose-specific, and restricted to predefined obligations within procurement agreements. Compliance with the Personal Data Protection Act (PDPA) is mandatory, particularly in defining explicit consent parameters, rules for data sharing between hospitals, payers, and pharmaceutical companies, and prohibitions on using data for marketing or unrelated research. Ethical data governance must also ensure strong privacy and cybersecurity

protections, including encryption, secure transmission layers, and role-based access to safeguard sensitive genetic information. Equally important is ensuring data quality and integrity, with standardized outcome measures — such as motor milestones, ventilator dependence, and hospitalization rates — collected consistently across institutions. External auditors or independent review committees should oversee data verification to prevent conflicts of interest and ensure neutrality. Given that gene therapy requires long-term monitoring, clear policies must define the duration of follow-up, continuity of registries when patients transfer hospitals, and sustainable funding mechanisms for maintaining national databases beyond pilot phases. FGDs consistently recommended establishing a national SMA registry to standardise data collection and ensure fairness in assessing real-world outcomes. Notably, 30% of KII participants expressed comfort with data sharing when it serves the common good, reflecting a willingness among families to contribute to collective learning, provided their children are not harmed or unfairly penalised. Ethical governance must therefore balance transparency and shared benefit with stringent protections to ensure that patient data is never misused, misinterpreted, or used to restrict access to therapy.

5.0 Recommendations

Eligibility Criteria

All eligibility criteria set for rare-disease treatments should be overseen by the Ministry of Health and determined by an unbiased group of experts to improve the outcomes and reach of the treatment. In the case of SMA treatment, a tiered eligibility framework should be adopted, coordinated and overseen by the Ministry of Health, that prioritises pre-symptomatic and early-diagnosed (<12 months, <3 SMN2 copies) patients for immediate access in the case of SMA. This would be supplemented by introducing second-tier eligibility for later-diagnosed patients, subject to clinical review, with partial subsidy or inclusion under an expanded access pathway.

Outcome Milestones

Successful outcomes for rare disease treatments should be redefined to reflect indicators beyond just clinically measured outcomes. Psychosocial factors and functional quality of life indicators should be included, with recommendations from patient groups taken into consideration. The established framework should combine clinical, functional, and patient-reported outcomes all within the same rubric to be measured concurrently. These outcomes are recommended to be overseen and reviewed by an independent outcomes review committee, eliminating bias in the process.

Health Systems and Governance

Malaysia should strengthen efforts made in the rare disease space through the development of a comprehensive framework for rare disease funding that would ensure sustainable and equitable access to high-cost therapies. Efforts to institutionalise a more structured and coordinated response to rare disease diagnosis and treatment should also be made in the health system to reduce fragmentation and streamline decision making. In line with this, the decision making system used should be transparent and kept accountable to build trust in the system and provide assurance to patients and caretakers alike. Greater health system awareness of rare diseases through training and the establishment of registries should also be carried out to build national capacity and awareness, as accessibility is crucial in the patient experience. While these are briefly addressed in the National Policy for Rare Diseases 2025, they should be detailed further with diseases-specific frameworks to become actionable.

Procurement

There is a growing need to pursue a more streamlined approach to outcome-based financing. In the near term, efforts should focus on expanding the budget allocated for rare diseases in the yearly budget cycle as well as looking at other forms of centralised funding.

Financing and Health Economics

Direct/pure financial payment streams should be put in place for established therapies with high upfront or one-off costs, as they offer a clear and predictable expenditure pattern, allowing healthcare systems to account for them more effectively. In the case of rare diseases and emerging therapies with uncertain long-term effectiveness, OBPMs should be explored, with clear frameworks in place to manage clinical and financial risk. Malaysia would greatly benefit from leveraging economics of scale through value-based approaches and regional collaboration to further improve access and affordability. This should be done in tandem with the long-term goal of integrating approved rare disease therapies as default inclusions in medical insurance policies, ultimately contributing to the establishment of a national medical insurance fund.

6.0 Conclusion

The introduction of single-dose, high-cost treatments such as gene therapy for SMA presents both an unprecedented opportunity and a profound policy challenge for Malaysia. This paper, grounded in evidence from international best practices, clinical expertise, and the lived experience of affected families, demonstrates that the status quo defined by prohibitive costs, fragmented access, and reliance on unsustainable charitable solutions is not an option for a nation committed to the principles of Malaysia MADANI and the UN Convention on the Rights of the Child.

This paper's central conclusion is that achieving sustainable and equitable access requires a fundamental paradigm shift: moving beyond the limitations of ad hoc charitable funding and toward a strategic, value-based, and systemic approach that includes payments streams and pooled funding. This shift is practical and fiscally viable. Specifically, we recommend that for established treatments such as onasemnogene abeparvovec (OA), the focus must be on reducing the financial burden through a pure financial payment stream (annuity model) to make government procurement manageable within existing budget allocations. Concurrently, the rigorous OBPM framework should be developed and reserved for future, high-uncertainty, high-cost therapies to ensure long-term fiscal accountability and managed risk.

Beyond the immediate financing mechanism, the durability of access depends on deep-seated reforms that create a resilient health ecosystem. These include the immediate establishment of data systems (National Registry, Newborn Screening), the embedding of ethical criteria that prioritise Quality of Life and social inclusion, and the institutionalisation of a process to fully assess and publish the financial profile of all emerging treatments. Critically, these efforts must be supported by the Ministry of Finance (MOF) and Bank Negara Malaysia (BNM) to explore macro-level financing solutions, particularly by deepening the insurance market and regulating default inclusion of rare disease coverage, leveraging the powerful economies of scale inherent in pooled funding.

Advancing access to gene therapy for SMA serves as a catalyst. The lessons learned in restructuring clinical criteria, managing procurement risk, and integrating patient-centric ethics can inform diagnosis, data systems, and financing mechanisms for all rare diseases. Ultimately, this policy framework is a call to action for a coordinated, whole-of-government response that not only ensures life-changing treatments reach every Malaysian child in need but also future-proofs the nation's health system for the inevitable wave of transformative medical innovation to come.

Appendix

Interview Participants

Table 2

Code	Description
S01	Parent of a child with SMA Type 2
S02	Parent of a child SMA Type 1
S03	Patient with SMA Type 2, with two siblings with the same type
S04	Parent of a child with SMA Type 2
S05	Parent of a child with SMA Type 2
S06	Parent of a child with SMA Type 1
S07	Parent of a child with SMA Type 1
S08	Parent of a child with SMA Type 1
Expert 1	Health economist with 17 years of experience in the industry
Expert 2	Insurance representative with 16 years of experience in the industry
Expert 3	Insurance representative with medical training and 12 years of working experience with the Ministry of Health

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