Needs and recommendations for policymakers

Policymakers can play a crucial role in these areas for persons with spinal muscular atrophy (PWSMA) and their caregivers.

"Fundamentally, the government needs to endorse national plans for rare diseases that provide frameworks incorporating a coordinated 'whole-of-government' approach to rare diseases, as opposed to a piecemeal approach. These plans should outline a cohesive clinical, public health and disability service approach to rare diseases that addresses prevention, timely diagnosis, early intervention, and appropriate access to treatments and rehabilitation."

– Living With Spinal Muscular Atrophy in Malaysia: A Study

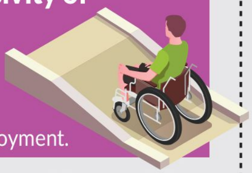
➢ Provide visibility and increase awareness of spinal muscular atrophy (SMA) at a national level.

This includes creating a national SMA registry.

"To them, some of us may look rather normal, but they don't know that we cannot even lift our hands."

– A woman in her 40s with SMA type 2

➢ Improve accessibility and promote inclusivity of PWSMA.



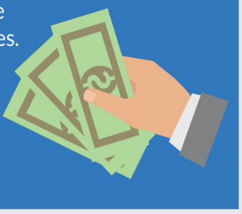
This includes ensuring equal opportunities for education and employment.

"I think the number one thing would be to make places more accessible. ... I mean, they have no idea how important it is - a small thing such as a ramp can make such a big difference, you know."

– A man in his 20s with SMA type 2

➢ Provide adequate resources for financial aid.

This includes providing an allocation in the annual Budget for PWSMA and outlawing genetic discrimination by insurance companies.



"The expense of purchasing assistive devices, mobility aids, respiratory support equipment (e.g. ventilators, BiPAP, CPAP and cough assist machine) and home modifications are exorbitant and are not covered by insurance, forcing some parents to find a second job, take out loans or look for charitable assistance."

– Living With Spinal Muscular Atrophy in Malaysia: A Study

"If you have a system that appreciates you as a human being, you won't be driven to the corner by people who don't know what or who you are. There will be no stigma because stigma has been eradicated by simple laws against discrimination."

– A woman in her 30s with SMA type 2

➢ Facilitate access to clinical trials and new drugs for SMA.

"My suggestion and top priority for the Government is to bring in Spinraza*. And put aside a certain allocation, you know, every year for SMA patients."

– A father of a boy with SMA Type 3

**At the time of interview, only Spinraza was approved for the treatment of SMA. Currently, there are two other drugs also approved for SMA treatment: Zolgensma and Evrysdi.*

➢ Ease the process of hiring personal living assistants for PWSMA.

This includes reducing the cost of hiring foreign domestic helpers who serve in such capacities.

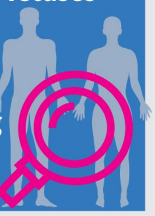
"This is my biggest concern, because if I can't find a helper, I would feel like I have lost both limbs and not be able to function."

– A woman in her 40s with SMA type 2

"Untreated SMA can have devastating consequences. Timely identification of symptoms and prompt referral to a specialised centre is critical to avoid a long diagnostic odyssey and enable earlier access to evidence-based care and intervention, as well as informing reproductive planning and reducing patient and parental stress."

– Living With Spinal Muscular Atrophy in Malaysia: A Study

➢ Enable a national screening process for SMA carriers (adults with one mutated copy of the SMN1 gene) and fetuses with SMA via prenatal diagnosis, carrier screening and/or newborn screening.



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