

Treatments that matter

These are the treatments that persons with spinal muscular atrophy (PWSMAs) and their caregivers consider the most important to them.

While SMA has no cure, there are a variety of treatments that can **improve the quality of life** of the PWSMA. Of crucial importance is to **diagnose the condition** and **start treatment as soon as possible** in order to best manage the complications of the disease.

“Untreated SMA can have devastating consequences.”

– Living With Spinal Muscular Atrophy in Malaysia: A Study

MOST IMPORTANT OUTCOMES FOR TREATMENT

- To provide gains in function
- To lessen symptoms
- To stop or slow down disease progression
- To prolong lifespan*

*Note: Only caregivers chose this option

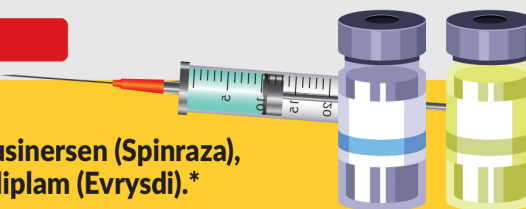
“When asked to indicate what would be the most important outcome for a possible drug treatment, providing gains in function, such as increased strength and energy, and being able to do something the PWSMA was unable to do before, were the most important outcomes for both PWSMA and caregivers.”

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CRUCIAL TREATMENT AREAS

DISEASE-MODIFYING TREATMENT

There are currently three drugs that can treat SMA: nusinersen (Spinraza), onasemnogene abeparvovec-xioi (Zolgensma) and risdiplam (Evrysdi).*



➤ Respiratory care

Lung disease is of primary concern as it is a main cause of death in SMA. The type of SMA will determine the extent of respiratory involvement. A proactive approach of introducing respiratory therapies early in the disease is the way forward in treating the pulmonary manifestations of SMA.”

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“Cough assist machine (CAM) has often enabled me to do my daily activities without being distracted by phlegm. Before I had started to use CAM, I was frequently struggling with phlegm in the morning as I always wake up with phlegm in lungs. ... Since I began to use CAM, I have been able to cough out my phlegm in the morning. It has assisted me to clear my lungs before I start the day. ... All in all, CAM has improved my lifestyle.”

– A man in his 20s with SMA type 2

➤ Rehabilitation and orthopaedic care

Rehabilitation goals differ according to the different types of SMA and the course of the disease, but these goals should also depend strongly on the patient's and family's wishes. ... Some of these goals include optimisation of function, minimisation of impairment and prevention of contractures and scoliosis.”

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➤ Palliative care

“I think palliative care is very, very important. The palliative team needs to come in and talk to the parents. Even if I know my child is going to pass on, what is the best I can do during this period? ... We have cases where parents who are given palliative consultation and all that, feel like, 'Okay, I have done my best, it's okay. That is the journey my child ... will finally go on.' If the parents are given that kind of counselling, besides genetic counselling, it gives a very good feeling, as if you are prepared, you have tried your best.”

– A mother of a boy with SMA Type 1

FOR CAREGIVERS, RESPITE CARE IS ALSO CRUCIAL

“We are very tired! And if the parents or the caregivers are not taken care of, it doesn't only affect the child with SMA, but the whole family. ... Our hope is to have support for caregivers like us. The Government or other NGOs can come in with a system that actually supports caregivers, like what they do in Australia, where maybe once in a week or once in a month, someone comes in to help relieve the caregivers, which I think is very, very important.”

– A mother of a boy with SMA Type 1

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