Plain English Summary

Drug treatments for spinal muscular atrophy

What does the guidance say?

Risdiplam is recommended for listing on the Medication Assistance Fund (MAF) for government subsidy for patients with spinal muscular atrophy (SMA) who meet certain clinical criteria.

Nusinersen is not recommended for subsidy for this condition.

What is spinal muscular atrophy?

Spinal muscular atrophy (SMA) is a rare, inherited condition (passed from parents to children) where a loss of specialised nerve cells (motor neurons) in the spinal cord causes weakness and wasting (atrophy) of muscles used for movement.

Motor neurons need a protein called survival motor neuron (SMN) protein to work properly. The SMN protein is made from two genes, SMN1 and SMN2. Patients with SMA do not have enough SMN protein due to changes (mutations) in the SMN1 gene. The SMN2 gene also produces SMN protein but at low levels.

The severity of SMA is associated with the number of SMN2 gene copies present. Fewer SMN2 gene copies are associated with more severe symptoms. The different types of SMA are also defined by the age when symptoms first appear and the physical milestones that the patient has reached:

- Type 1 SMA is the most common form and affects infants less than 6 months old. Patients experience severe weakness and cannot sit unassisted. They can also have difficulties breathing and swallowing, and poor head control. Without treatment, many patients may not survive beyond 2 years of age due to respiratory failure.
- Type 2 SMA affects babies between six and 18 months old and is less severe than Type 1 SMA. Individuals can usually sit without support but cannot stand or walk unaided. They may also have respiratory muscle weakness which can be life-threatening.
- Type 3 SMA affects children and young people between 18 months and 18 years old. Most individuals can walk unaided but some may require wheelchair assistance as their motor function declines over time.

Plain English Summary

Drug treatments for spinal muscular atrophy

What is risdiplam?

Risdiplam belongs to a group of medicines called SMN2 splicing modifiers that target the SMN2 gene to make more SMN protein. It is taken orally. Your doctor or your child's doctor will tell you how much to take and how long to take it for.

Who can have risdiplam?

Risdiplam can be used by patients with Type 1, 2 or 3 SMA who have symptoms and:

- are or were 18 years old or younger when they started drug treatment for SMA; or
- are at least 19 years old, and had not started drug treatment for SMA before they turned 19 years old despite showing symptoms.

Risdiplam can also be used by patients with SMA:

- who have not shown symptoms, and are or were younger than 3 years old when they started drug treatment for SMA; or
- whose condition has worsened despite receiving gene therapy for SMA.

Your doctor or your child's doctor can advise if risdiplam is a suitable treatment.

Why was risdiplam recommended for subsidy?

ACE evaluates how well a treatment works in relation to how much it costs compared to other treatments. Risdiplam was recommended for subsidy for certain patients with SMA as it was considered to be an acceptable use of healthcare resources at the price proposed by the company.

Nusinersen was not recommended for subsidy because its benefits do not justify its cost compared with risdiplam. If you or your child needs nusinersen for SMA, you can speak to a medical social worker to find out if there is other financial assistance available to help with the cost of treatment.

Plain English Summary

Drug treatments for spinal muscular atrophy

What does listing on the MAF mean for me?

The MAF helps people pay for treatments that are clinically effective and cost effective. If your doctor or your child's doctor prescribes risdiplam in line with the MAF criteria, the treatment cost will be subsidised by 40% to 75%.

Published: 4 June 2024

Agency for Care Effectiveness - ACE Agency for Care Effectiveness (ACE)

The Agency for Care Effectiveness (ACE) was established by the Ministry of Health (Singapore) to drive better decision-making in healthcare through health technology assessment (HTA), clinical guidance and education. It publishes guidances on diagnosing, treating, and preventing different medical conditions based on the latest research information available worldwide.

This summary is not, and should not be regarded as, a substitute for professional or medical advice. Please seek the advice of a qualified healthcare professional about any medical condition.

© Agency for Care Effectiveness, Ministry of Health, Republic of Singapore
All rights reserved. Reproduction of this publication in whole or part in any material form is prohibited without the prior written permission of the copyright holder.

To find out more about ACE visit www.ace-hta.gov.sg