

## Plain English Summary

# Gene therapy for spinal muscular atrophy

### What does the guidance say?

Onasemnogene abeparvovec is not recommended for inclusion on the MOH Cell, Tissue and Gene Therapy Product (CTGTP) List for government funding for children with spinal muscular atrophy.

### What is spinal muscular atrophy?

Spinal muscular atrophy (SMA) is a rare, inherited condition 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. “Gene copies” refer to how many of the same gene a person has. Having more SMN2 gene copies means the body can produce more SMN protein, which helps reduce the severity of symptoms. Patients with fewer SMN2 gene copies make less SMN protein and are more likely to have severe symptoms. There are different types of SMA defined by the age when symptoms first appear and the motor function of the patient.

### What is onasemnogene abeparvovec?

Onasemnogene abeparvovec is a gene therapy. It replaces the faulty SMN1 gene and increases the amount of SMN protein in the body to keep the muscles working. This one-time treatment is given as a slow drip into a vein (intravenously).

### Why was it not recommended for funding?

ACE evaluates how well a treatment works in relation to how much it costs compared to other treatments. Onasemnogene abeparvovec was not recommended for government funding because its long-term benefits for patients with SMA compared to alternative treatment options are uncertain and do not justify its higher cost. If your child needs onasemnogene abeparvovec for this condition, you can speak to a medical social worker to find out if there is financial assistance available to help with the cost of treatment.

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### Is this the right treatment for my child?


There are different types of treatments available for SMA. Your child's doctor may recommend onasemnogene abeparvovec if they consider that it is the most suitable treatment for your child's condition. Your child's doctor should give you clear information, listen to your views and concerns, and talk to you about your child's treatment options.

Some of the questions you may want to ask your child's doctor when making decisions about your child's care are:

- How will the treatment affect my child's day-to-day activities?
- How well does it work compared to other treatments?
- What are the side effects and risks of treatment, and how likely are they?
- How much does the treatment cost, and who can I approach if I want to find out whether there is financial assistance available to help with the cost of treatment?
- What happens if the treatment stops working?
- What happens if my child does not have the treatment?

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 Agency for Care Effectiveness - ACE

 Agency for Care Effectiveness (ACE)

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