Assessment explained

Medicine: velmanase alfa (brand name: Lamzede®) for alpha mannosidosis

Chiesi Limited

Velmanase alfa meets the Scottish Medicines Consortium (SMC) definition of an ultra-orphan medicine, which is a medicine to treat an extremely rare condition. This document summarises the initial SMC assessment of velmanase alfa for treating mild to moderate mannosidosis. It is used for treating effects of the condition that do not involve the brain (non-neurological manifestations).

What does this mean for patients?

If your healthcare professional thinks that velmanase alfa is the right medicine for you or your child, you or they should be able to have the treatment on the NHS in Scotland within the **ultra-orphan pathway** (see next page). This is provided the company submits a plan to the Scottish Government describing how further data, including on the patient and carer lived experience, will be collected over the next 3 years. After this, SMC will reassess the medicine and make a decision on routine availability.

What is velmanase alfa used for?

Velmanase alfa used to treat patients with alpha mannosidosis, which is a rare inherited condition. Alpha mannosidosis causes a range of symptoms including: difficulty controlling movement, breathing problems, deafness, speech difficulties, infections, bone abnormalities, muscle pain and weakness and learning disabilities. Velmanase alfa is used for treating patients with mild to moderate alpha mannosidosis. It is used for treating the effects of the condition that do not involve the brain (non-neurological manifestations).

How does velmanase alfa work?

Patients with alpha mannosidosis have a shortage of an enzyme called alpha mannosidase. This causes a type of sugar called oligosaccharides to build up and cause damage to different tissues in the body. Velmanase alfa is a replacement for alpha mannosidase and acts in the same way. By doing this it helps prevent the build-up of the oligosaccharides and the worsening of the symptoms of alpha mannosidosis.

How do we assess ultra-orphan medicines?

SMC uses a broad assessment framework for ultra-orphan medicines. This is part of the ultra-orphan pathway in NHSScotland which has **four stages**:

Stage 1 Validation

SMC reviews information from the company to decide if the ultra-orphan definition is met

The company must then agree to further requirements of the ultra-orphan pathway*.

Stage 2 Initial Assessment

SMC considers the:

- nature of the condition
- health benefits of the medicine
- impact beyond direct health benefits and on specialist services
- value for money, and
- costs to the NHS.

Stage 3 Evidence Generation

The medicine can be prescribed while the company gathers futher data, including on the patient and carer lived experience.

Stage 4 Reassessment

After 3 years the company provides an updated submission for reassessment.

SMC considers all the evidence and makes a decision on routine use of the medicine in NHSScotland.

*provide a confidential discount known as a Patient Access Scheme (PAS) to increase the cost-effectiveness of the medicine, and provide a data collection plan.

What have we said in this assessment?

- Data showed that after 52 weeks velmanase alfa reduced levels of oligosaccharides in the blood of treated patients compared with placebo (a dummy treatment). There were no significant differences between the two groups in quality of life measurements.
- There were some limitations in the study and the 52 weeks was considered too short to properly assess the longer term benefits of velmanase alfa on disease progression and complications as well as long term safety.
- Despite the confidential discount offered by the company, the cost in relation to the health benefits of velmanase alfa remains high.

For further information please see the SMC ultra-orphan medicine initial assessment report (SMC2466).

More information

The organisation below can provide more information and support for people with alpha mannosidosis and their families. SMC is not responsible for the content of any information provided by external organisations.

MPS Society

https://www.mpssociety.org.uk 0345 389 9901

You can find out more about velmanase alfa (Lamzede®) in the Patient Leaflet (PIL) by searching for the medicine name on the electronic medicines compendium (EMC) website.

https://www.medicines.org.uk/emc/