Plain English Summary

Enzyme replacement therapies for treating Fabry disease

What does the guidance say?

Agalsidase alfa is recommended for listing on the Medication Assistance Fund (MAF) for government subsidy for people with Fabry disease who meet certain clinical criteria.

Agalsidase beta is not recommended for subsidy for this condition.

What is Fabry disease?

Fabry disease is a rare, progressive, inherited lysosomal storage disorder. Cells in the body have structures called lysosomes which contain enzymes that can break down substances for the cell to use or remove as waste. People with Fabry disease do not produce enough of the enzyme alpha-galactosidase A, leading to a build-up of waste products in the lysosome which affects its function.

There are two main types of Fabry disease. The most severe form, also known as classical Fabry disease, typically begins in childhood, mainly in males who have no enzyme activity. Milder forms, or non-classical Fabry disease, occur later in life in male and female patients who have some enzyme activity.

Symptoms of Fabry disease include episodes of pain in the hands and feet, small; dark red spots on the skin; a reduced ability to sweat; cloudiness in the front part of the eye; gastrointestinal symptoms; and hearing loss. As the disease progresses, it can affect many parts of the body and may lead to serious complications such as kidney failure, heart attack and stroke.

What is agalsidase alfa?

Agalsidase belongs to a group of medicines called enzyme replacement therapy. There are two types: agalsidase alfa and agalsidase beta, and both types are synthetic (man-made) forms of natural human alpha-galactosidase A. They are used to replace the deficient level of alpha-galactosidase A in the cells and reduce the build-up of waste to prevent complications and organ failure.

Agalsidase alfa is given as a slow drip into a vein (intravenously). Your doctor or your child's doctor will tell you how much you need to have and how long you need to have it for.

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Who can have agalsidase alfa?

Agalsidase alfa can be used as an enzyme replacement therapy by:

- male patients with classical Fabry disease; or
- male patients with non-classical Fabry disease, or female patients with classical or non-classical Fabry disease, who have signs and symptoms that their kidneys, heart or nervous system are being affected by their condition, that cannot be explained by other causes.

Agalsidase alfa should be stopped if the disease worsens while on treatment. Your doctor or your child's doctor can advise if agalsidase alfa is a suitable treatment.

Why was agalsidase alfa recommended for subsidy?

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

Agalsidase beta was not recommended for subsidy because its benefits do not justify its cost compared with agalsidase alfa. If you need agalsidase beta for this condition, 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.

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 agalsidase alfa in line with the MAF criteria, the treatment cost will be subsidised by 40% to 75%.

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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.

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