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**EPAR summary for the public**

# Fabrazyme

agalsidase beta

This is a summary of the European public assessment report (EPAR) for Fabrazyme. It explains how the Committee for Medicinal Products for Human Use (CHMP) assessed the medicine to reach its opinion in favour of granting a marketing authorisation and its recommendations on the conditions of use for Fabrazyme.

## What is Fabrazyme?

Fabrazyme is a medicine that contains the active substance agalsidase beta. It is available as a powder to be made into a solution for infusion (drip) into a vein.

## What is Fabrazyme used for?

Fabrazyme is used to treat patients who have Fabry disease, a rare inherited disorder. Patients with Fabry disease do not have enough of an enzyme called alpha-galactosidase A. This enzyme normally breaks down a fatty substance called globotriaosylceramide (GL-3 or Gb3). If the enzyme is not present, GL-3 cannot be broken down and it builds up in the body's cells, such as kidney cells.

People with Fabry disease may have a wide range of signs and symptoms, including severe conditions such as kidney failure, heart problems and stroke.

The medicine can only be obtained with a prescription.

## How is Fabrazyme used?

Only a doctor who has experience in treating patients with Fabry disease or other inherited metabolic diseases should give Fabrazyme.

Fabrazyme is given once every two weeks as an infusion of 1 mg per kilogram body weight. The starting infusion rate should be no more than 0.25 mg per minute (15 mg per hour) to reduce the risk of infusion-related side effects. The infusion rate may be increased gradually with further infusions.

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Fabrazyme is intended for long-term use. The infusions are given in hospital but may be given at home if it has been shown that the patient is tolerating the infusions well.

## How does Fabrazyme work?

Fabrazyme is an enzyme replacement therapy. Enzyme replacement therapies provide patients with the enzyme they are lacking. Fabrazyme is designed to replace the human enzyme alpha galactosidase A, which is lacking in patients with Fabry disease. The active substance in Fabrazyme, agalsidase beta, is a copy of the human enzyme, produced by a method known as 'recombinant DNA technology': it is made by cells that have received a gene (DNA), which makes them able to produce the enzyme. The replacement enzyme helps to break down GL-3 and stops it building up in the patient's cells.

## How has Fabrazyme been studied?

Fabrazyme has been investigated in three studies involving a total of 73 adults. In the main study, Fabrazyme was compared with placebo (a dummy treatment) in 58 patients. The study looked at the effects of the medicine on clearing GL-3 from the kidney. The effectiveness of Fabrazyme was also tested in 16 children aged between eight and 16 years who had Fabry disease.

## What benefit has Fabrazyme shown during the studies?

In the main study, Fabrazyme produced a highly significant and almost complete clearance of GL-3 in the kidney cells after 20 weeks of treatment: 69% of the patients treated with Fabrazyme had the best score for clearance, compared with none of the patients in the placebo group.

Children treated with Fabrazyme also had decreases in levels of GL-3 in the blood, with all children having normal levels after 20 weeks of treatment. This was accompanied by improvements in symptoms and quality of life.

## What is the risk associated with Fabrazyme?

The most common side effects with Fabrazyme (seen in more than 1 patient in 10) are caused by the infusion rather than the medicine. These include fever, chills, headache, paraesthesia (abnormal sensations like pins and needles), nausea (feeling sick), vomiting and feeling cold. For the full list of all side effects reported with Fabrazyme, see the package leaflet.

Fabrazyme must not be used in people who are hypersensitive (allergic) to agalsidase beta or any of the other ingredients.

## Why has Fabrazyme been approved?

The CHMP decided that, for patients with Fabry disease, treatment with Fabrazyme might provide long-term clinical benefits. The CHMP decided that Fabrazyme's benefits are greater than its risks and recommended that it be given marketing authorisation.

## Other information about Fabrazyme

The European Commission granted a marketing authorisation valid throughout the European Union for Fabrazyme on 3 August 2001.

The full EPAR for Fabrazyme can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European Public Assessment Reports. For more information about

treatment with Fabrazyme, read the package leaflet (also part of the EPAR) or contact your doctor or pharmacist.

This summary was last updated in 02-2013.