EMA/450563/2021
EMEA/H/C/004019

Ucedane (*carglumic acid*)

An overview of Ucedane and why it is authorised in the EU

What is Ucedane and what is it used for?

Ucedane is a medicine used for the treatment of hyperammonaemia (high blood levels of ammonia) in patients with the following metabolic diseases:

- N-acetylglutamate synthase (NAGS) deficiency. Patients with this lifelong disease lack a liver enzyme called NAGS, which normally helps to break down ammonia. If the enzyme is not present, ammonia cannot be broken down and it builds up in the blood;
- some organic acidaemias (isovaleric acidaemia, methylmalonic acidaemia and propionic acidaemia) where patients lack certain enzymes involved in protein metabolism.

Ucedane contains the active substance carglumic acid and is a 'generic medicine'. This means that Ucedane contains the same active substance and works in the same way as a 'reference medicine' already authorised in the European Union (EU) called Carbaglu. For more information on generic medicines, see the question-and-answer document here.

How is Ucedane used?

Ucedane is available as dispersible tablets (200 mg) that are to be dispersed (mixed) in a small amount of water. The medicine can only be obtained with a prescription and treatment should be started by a doctor who has experience in treating patients with metabolic diseases.

In patients with NAGS deficiency, treatment may be started as early as the first day of life and the medicine is used for the patient's whole life. In patients with organic acidaemias, treatment is started when the patient has a hyperammonaemia crisis and continued until the crisis is finished.

The initial daily dose of Ucedane should be 100 mg per kilogram body weight, but up to 250 mg/kg can be used if necessary. The dose should then be adjusted to maintain normal blood ammonia levels.

For more information about using Ucedane, see the package leaflet or contact your doctor or pharmacist.

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How does Ucedane work?

When ammonia builds up in the blood, it is toxic to the body, especially the brain. The active substance in Ucedane, carglumic acid, is very similar in structure to N-acetylglutamate, which activates an enzyme that breaks down ammonia. Ucedane therefore helps break down ammonia, reducing ammonia blood levels and its toxic effects.

How has Ucedane been studied?

Studies on the benefits and risks of the active substance in the approved use have already been carried out with the reference medicine, Carbaglu, and do not need to be repeated for Ucedane.

As for every medicine, the company provided studies on the quality of Ucedane. The company also carried out a study that showed that it is 'bioequivalent' to the reference medicine. Two medicines are bioequivalent when they produce the same levels of the active substance in the body and are therefore expected to have the same effect.

What are the benefits and risks of Ucedane?

Because Ucedane is a generic medicine and is bioequivalent to the reference medicine, its benefits and risks are taken as being the same as the reference medicine's.

Why is Ucedane authorised in the EU?

The European Medicines Agency concluded that, in accordance with EU requirements, Ucedane has been shown to have comparable quality and to be bioequivalent to Carbaglu. Therefore, the Agency's view was that, as for Carbaglu, the benefits of Ucedane outweigh the identified risk and it can be authorised for use in the EU.

What measures are being taken to ensure the safe and effective use of Ucedane?

Recommendations and precautions to be followed by healthcare professionals and patients for the safe and effective use of Ucedane have been included in the summary of product characteristics and the package leaflet.

Other information about Ucedane

Ucedane received a marketing authorisation valid throughout the EU on 23 June 2017.

Further information on Ucedane can be found on the Agency's website: ema.europa.eu/medicines/human/EPAR/ucedane

This overview was last updated in 08-2021.