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EPAR summary for the public

Ucedane

carglumic acid

This is a summary of the European public assessment report (EPAR) for Ucedane. It explains how the Agency assessed the medicine to recommend its authorisation in the EU and its conditions of use. It is not intended to provide practical advice on how to use Ucedane.

For practical information about using Ucedane, patients should read the package leaflet or contact their doctor or pharmacist.

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 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.

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 such as 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.

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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.

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 approved?

The Agency's Committee for Medicinal Products for Human Use (CHMP) concluded that, in accordance with EU requirements, Ucedane has been shown to have comparable quality and to be bioequivalent to Carbaglu. Therefore, the CHMP's view was that, as for Carbaglu, the benefit outweighs the identified risk. The Committee recommended that Ucedane be approved 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

The European Commission granted a marketing authorisation valid throughout the European Union for Ucedane on 23 June 2017.

The full EPAR for Ucedane 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 Ucedane, read the package leaflet (also part of the EPAR) or contact your doctor or pharmacist.

The full EPAR for the reference medicine can also be found on the Agency's website.

This summary was last updated in 06-2017.