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Maapliv (*L-alanine, L-arginine, L-aspartic acid, L-cysteine hydrochloride monohydrate, L-glutamic acid, glycine, L-histidine, L-lysine monohydrate, L-methionine, L-phenylalanine, L-proline, L-serine, taurine, L-threonine, L-tryptophan, L-tyrosine*)

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

What is Maapliv and what is it used for?

Maapliv is a medicine used to treat an acute decompensation episode (sudden worsening) of maple syrup urine disease. Maple syrup urine disease is an inherited disorder in which the body is unable to break down certain building blocks of protein known as branched-chain amino acids (BCAAs), including leucine, isoleucine and valine. As a result, these amino acids build up in the body, including in the brain, where leucine in particular can lead to brain damage.

Maapliv is for use in patients who cannot receive BCAA-free amino acids by mouth or via a feeding tube.

Maple syrup urine disease is rare, and Maapliv was designated an 'orphan medicine' (a medicine used in rare diseases) on 26 October 2018. Further information on the orphan designation can be found on the EMA website.

Maapliv contains the following amino acids as active substance: L-alanine, L-arginine, L-aspartic acid, L-cysteine hydrochloride monodhydrate, L-glutamic acid, glycine, L-histidine, L-lysine monohydrate, L-methionine, L-phenylalanine, L-proline, L-serine, taurine, L-threonine, L-tryptophan, L-tyrosine.

How is Maapliv used?

Maapliv can only be obtained with a prescription, and treatment should be started under the supervision of a doctor experienced in managing maple syrup urine disease.

The medicine is given as an infusion (drip) into a vein until the leucine levels have returned to normal.

In patients who have severe or critical worsening of disease and very high levels of leucine, treatment with Maapliv may not be enough to lower the leucine levels and they may need other treatments like haemodialysis or haemofiltration (procedures to remove waste products from the blood).

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

How does Maapliv work?

Maapliv consists of a solution of amino acids, but with no BCAAs. When Maapliv is given to the patient, the body uses the amino acids to make proteins, together with the excess BCAAs that have built up because of the sudden disease worsening. This lowers the level of BCAAs in the body, thereby reducing their toxic effect.

What benefits of Maapliv have been shown in studies?

The company provided data from 5 studies published in the scientific literature. The studies involved a total of 88 patients with maple syrup urine disease and looked at the use of a BCAA-free mixture of amino acids to treat sudden worsening of the disease based on high levels of leucine. The amino acids were the same as those in Maapliv and were given either as an infusion or by mouth or feeding tube. The studies found that BCAA-free amino acids mixtures reduced the levels of leucine to normal by the time patients left the hospital.

What are the risks associated with Maapliv?

For the full list of side effects and restrictions with Maapliv, see the package leaflet.

The side effects of BCAA-free amino acid mixtures reported in the literature (which may affect up to 1 in 100 people) include anaphylactic or anaphylactoid reactions (sudden, severe allergic reaction), hypersensitivity (allergic reaction), hyperammonaemia (high blood levels of ammonia), pulmonary thromboembolism (blood clot in a blood vessel in the lungs), venous thromboembolism (blood clot in a vein), respiratory distress (breathing difficulties), increased blood levels of bilirubin (a breakdown product of red blood cells), increased levels of liver enzymes (a sign of problems with liver function), cholestasis (problems with bile flow), cholecystitis (inflammation of the gallbladder caused by trapped bile), cholelithiasis (gallstones; hardened pieces of bile in the gallbladder), azotaemia (high blood levels of nitrogen waste products, a sign of problems with kidney function), infusion site thrombophlebitis (inflammation of the vein receiving the drip) and venous irritation (irritation of the vein receiving the drip).

Why is Maapliv authorised in the EU?

Maple syrup urine disease is a rare disease with an unmet medical need. At the time of Maapliv authorisation, there were no medicines authorised to treat either the disease itself or episodes of sudden worsening of the disease, which require urgent treatment. Maapliv is a ready-made mixture of BCAA-free amino acids that can be given by infusion. Studies have shown that BCAA-free amino acids such as Maapliv can bring the level of leucine back down to normal in patients experiencing a sudden worsening of their disease. Normalisation of leucine levels is an important part of managing maple syrup urine disease. Giving the medicine by infusion also provides a benefit for patients who cannot take treatment by mouth or feeding tube. In terms of safety, treatment with BCAA-free amino acids was well tolerated and there were no safety concerns based on the available data.

Maapliv has been authorised under ‘exceptional circumstances’. This is because it has not been possible to obtain complete information about Maapliv due to the rarity of the disease. The company must provide further data on Maapliv. It must carry out a study looking at the effectiveness and safety of Maapliv in treating acute decompensation episodes in patients with maple syrup urine disease. The company will also provide yearly updates on any new information relating to the medicine’s safety and

effectiveness. Every year, the European Medicines Agency will review any new information that becomes available.

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

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

As for all medicines, data on the use of Maapliv are continuously monitored. Suspected side effects reported with Maapliv are carefully evaluated and any necessary action taken to protect patients.

Other information about Maapliv

Maapliv received a marketing authorisation under exceptional circumstances valid throughout the EU on 28 July 2025

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

This overview was last updated in 06-2025.