22 May 2025
EMA/CHMP/164305/2025
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Maapliv

amino acids

On 22 May 2025, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation under exceptional circumstances[2] for the medicinal product Maapliv[3], intended for the treatment of maple syrup urine disease (MSUD). The applicant for this medicinal product is Recordati Rare Diseases.

Maapliv will be available as a solution for infusion. The active substance of Maapliv is a combination of amino acids free of branched-chain amino acids (BCAA), and the medicine is a solution for parenteral use (ATC code: B05BA01). Maapliv, in combination with carbohydrate and lipid supplementation, prevents or reverses protein catabolism and promotes anabolism in patients with MSUD decompensation, thereby reducing alpha-keto acid levels.

The benefit of Maapliv is leucine normalisation in patients with MSUD decompensation as shown in five scientific publications which report on parenteral use of BCAA-free solutions with the same formulation as Maapliv. The parenteral use of BCAA-free solutions in these publications was associated with uncommon side effects, which are provided in the summary of product characteristics (SmPC).

The full indication is:

> Maapliv is indicated for the treatment of maple syrup urine disease (MSUD) presenting with an acute decompensation episode in patients from birth who are not eligible for an oral and enteral branched-chain amino acids free (BCAA- free) formulation.

Treatment with Maapliv should initiated under the supervision of a physician experienced in the management of MSUD disease.

Detailed recommendations for the use of this product will be described in the SmPC, which will be published on the EMA website in all official European Union languages after the marketing authorisation

[1] Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion
[2] In exceptional circumstances, an authorisation may be granted subject to certain specific obligations, to be reviewed annually. This happens when the applicant can show that they are unable to provide comprehensive data on the efficacy and safety of the medicinal product, due to the rarity of the condition it is intended for, limited scientific knowledge in the area concerned, or ethical considerations involved in the collection of such data
[3] This product was designated as an orphan medicine during its development. EMA will now review the information available to date to determine if the orphan designation can be maintained

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has been granted by the European Commission.