25 July 2024
EMA/CHMP/314863/2024
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Kayfanda

odevixibat

On 25 July 2024, 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 Kayfanda, intended for the treatment of cholestatic pruritus in patients with Alagille Syndrome (ALGS) in patients aged 6 months or older.

The applicant for this medicinal product is Ipsen Pharma.

Kayfanda will be available as 200 µg, 400 µg, 600 µg and 1200 µg hard capsules. The active substance of Kayfanda is odevixibat, a bile therapy (ATC code: A05AX05). Odevixibat is a reversible and selective inhibitor of the ileal bile acid transporter (IBAT) that acts locally in the distal ileum. It reduces the reuptake of bile acids and increases the clearance of bile acids through the colon.

The benefits of Kayfanda are its clinically relevant effects on pruritus and associated sleep disturbances in ALGS patients. In addition, Kayfanda reduces the concentration of serum bile acids of patients with ALGS. The most common side effects with Kayfanda are diarrhoea, abdominal pain, vomiting, and hepatic enzymes increased.

The full indication is:

> Kayfanda is indicated for the treatment of cholestatic pruritus in Alagille syndrome (ALGS) in patients aged 6 months or older (see sections 4.4 and 5.1).

Treatment with Kayfanda must be initiated and supervised by physicians experienced in the management of ALGS.

Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published in the European public assessment report (EPAR) and made available in all official European Union languages after the marketing authorisation has been

[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.

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