20 May 2021
EMA/CHMP/273563/2021
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Bylvay

odevixibat

On 20 May 2021, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation under exceptional circumstances[3] for the medicinal product Bylvay[2], intended for the treatment of progressive familial intrahepatic cholestasis (PFIC) in patients aged 6 months or older.

Bylvay was reviewed under EMA's accelerated assessment programme.

The applicant for this medicinal product is Albireo.

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

The benefit of Bylvay is its ability to reduce the concentration of bile acids in the serum of patients with PFIC. The most common side effects are diarrhoea, abdominal pain, haemorrhagic diarrhoea, soft faeces, and hepatomegaly.

The full indication is:

> Treatment of progressive familial intrahepatic cholestasis (PFIC) in patients aged 6 months or older.

Bylvay treatment must be initiated and supervised by physicians experienced in the management of PFIC.

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 granted by the European Commission.

[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] 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

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

[3] 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.