



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

13 October 2022  
EMA/CHMP/803191/2022  
Committee for Medicinal Products for Human Use (CHMP)

## Summary of opinion<sup>1</sup> (initial authorisation)

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### Livmarli maralixibat chloride

On 13 October 2022, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation under exceptional circumstances<sup>2</sup> for the medicinal product Livmarli<sup>3</sup>, intended for the treatment of cholestatic pruritus in patients with Alagille syndrome (ALGS).

The applicant for this medicinal product is Mirum Pharmaceuticals International B.V.

Livmarli will be available as an oral solution. The active substance of Livmarli is maralixibat chloride, a potent, reversible, selective inhibitor of the ileal bile acid transporter (ATC code: A05AX04). Each ml of solution contains maralixibat chloride equivalent to 9.5 mg maralixibat. Maralixibat acts locally in the distal ileum to decrease the reuptake and increase the clearance of bile acids through the colon, thereby reducing their concentration in the serum.

The benefit of Livmarli is its ability to reduce serum bile acid-related pruritus in patients with ALGS. The most common side effects are diarrhoea and abdominal pain.

The full indication is:

Livmarli is indicated for the treatment of cholestatic pruritus in patients with Alagille syndrome (ALGS) 2 months of age and older.

Treatment with Livmarli should be initiated under the supervision of a physician experienced in the management of patients with cholestatic liver diseases.

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

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<sup>1</sup> Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion

<sup>2</sup> 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.

<sup>3</sup> 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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granted by the European Commission.