29 January 2026
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Committee for Medicinal Products for Human Use (CHMP)

Scientific conclusions and grounds for the variation to the terms of the marketing authorisation(s)

Active substance(s): lumacaftor / ivacaftor

Procedure No. PSUSA/00010455/202505

Period covered by the PSUR:
1 year to 19 May 2025

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Scientific conclusions

Taking into account the PRAC Assessment Report on the PSUR(s) for lumacaftor / ivacaftor, the scientific conclusions of PRAC are as follows:

In view of cumulative available data on cases of liver failure, in the context of updates made to the product information for elexacaftor/tezacaftor/ivacaftor, given the very serious nature of the events in question, the PRAC concluded that the product information of products containing lumacaftor/ivacaftor should be amended accordingly.

In view of available data on anxiety and insomnia from post-marketing reports including in some cases a positive de-challenge in the context of updates made to the product information for elexacaftor/tezacaftor/ivacaftor, the PRAC Rapporteur considers a causal relationship between lumacaftor/ivacaftor and anxiety and insomnia is at least a reasonable possibility. The PRAC concluded that the product information of products containing lumacaftor/ivacaftor should be amended accordingly.

Having reviewed the PRAC recommendation, the CHMP agrees with the PRAC overall conclusions and grounds for recommendation.

Grounds for the variation to the terms of the marketing authorisation(s)

On the basis of the scientific conclusions for lumacaftor / ivacaftor the CHMP is of the opinion that the benefit-risk balance of the medicinal product(s) containing lumacaftor / ivacaftor is unchanged subject to the proposed changes to the product information.

The CHMP recommends that the terms of the marketing authorisation(s) should be varied.