25 January 2024
EMA/86623/2024
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): onasemnogene abeparvovec

Procedure No. EMEA/H/C/PSUSA/00010848/202305

Period covered by the PSUR: 24 May 2022 To: 23 May 2023

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Scientific conclusions

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

In view of available data on hepatotoxicity, TMA and thrombocytopenia from clinical studies, the literature, and spontaneous reports, the PRAC concluded that the product information of products containing onasemnogene abeparvovec 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 onasemnogene abeparvovec the CHMP is of the opinion that the benefit-risk balance of the medicinal product(s) containing onasemnogene abeparvovec 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.