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EMA confirms suspension of sickle cell disease medicine Oxbryta

Higher rate of death and disease complications in recent trials means benefit-risk balance no longer favourable

EMA's human medicines committee (CHMP) has recommended that the marketing authorisation of the sickle cell disease medicine Oxbryta remain suspended. This recommendation follows interim measures taken by the Committee in September 2024, when it temporarily suspended the medicine to review emerging safety data.

Following its assessment, the CHMP concluded that the benefits of the medicine no longer outweigh its risks. The review was started after data showed a higher number of deaths with Oxbryta than with placebo (a dummy treatment) in one clinical trial[1] and a higher-than-anticipated number of deaths in another trial.[2]

In the first study, which assessed the effect of Oxbryta in people with sickle cell disease who were at higher risk of stroke, 8 children treated with Oxbryta died, compared with 2 children who received a placebo treatment. In the second study, which evaluated the effect of the medicine on leg ulcers, a known complication of sickle cell disease, 1 person died in the Oxbryta group during the first 12 weeks of treatment, while no deaths occurred in the placebo group. In the following 12-week phase of the study, where all patients received Oxbryta, 8 additional deaths were reported. The studies also showed a higher number of sudden episodes of severe pain, including vaso-occlusive crises (VOC) among patients treated with Oxbryta compared to those receiving placebo.

On 26 September 2024, the CHMP recommended suspending the marketing authorisation as a precautionary measure when further data from two registry-based studies emerged, indicating that patients experienced a higher frequency of sudden pain episodes with Oxbryta than before starting treatment. At the time, EMA provided advice to healthcare professionals and patients, highlighting that

[1] Global Blood Therapeutics. *GBT440-032 Phase 3 Study in Participants with Sickle Cell Disease (HOPE Kids 2)*. IRAS ID: 242661. EudraCT Number: 2017-000903-26. REC Reference: 18/LO/0359. London - City & East Research Ethics Committee. Health Research Authority. Available at: https://www.hra.nhs.uk/planning-and-improving-research/application-summaries/research-summaries/gbtt440-032-phase-3-study-in-participants-with-sickle-cell-disease

[2] *A Phase 3, Multicenter, Randomized, Double-Blind, Placebo-Controlled Trial to Evaluate the Efficacy of Voxelotor for the Treatment of Leg Ulcers in Patients with Sickle Cell Disease (RESOLVE)*. EudraCT Number: 2025-000161-87. ClinicalTrials.gov Identifier: NCT05561140. Available at: https://www.clinicaltrialsregister.eu/ctr-search/trial/2025-000161-87/3rd

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Oxbryta should no longer be prescribed and that existing patients should be switched to an alternative treatment.[3]

Although the final analysis of the registry studies did not confirm an increase in sudden pain episodes with Oxbryta, the recent clinical trials did show more sudden pain episodes and deaths. These results are inconsistent with those of the earlier main clinical trial that supported Oxbryta's authorisation, which had not shown a difference between treatment groups.

In its review, the CHMP noted that the underlying mechanisms for the increased number of deaths and complications, including sudden pain episodes, following treatment with Oxbryta in the studies remain unclear. The CHMP found no clear explanation for the increased risks and could not identify measures to effectively minimise these risks or any subgroup of patients for whom the medicine's benefits would outweigh its risks. As a result, the CHMP concluded that the benefit-risk balance of Oxbryta is no longer favourable, and the suspension of the medicine's marketing authorisation should remain in place. Oxbryta will therefore remain unavailable for healthcare professionals to prescribe to patients in the EU.

In reaching its opinion, the CHMP also took into account advice from experts in the field as well as from patient representatives and consulted EMA's safety committee (PRAC) on potential risk minimisation measures.

More about the medicine

Oxbryta was authorised in February 2022 to treat haemolytic anaemia (excess breakdown of red blood cells) in patients aged 12 years and older who have sickle cell disease. It was given on its own or together with another medicine for sickle cell disease called hydroxycarbamide. Oxbryta contains the active substance voxelotor.

Sickle cell disease is a genetic disease where individuals produce an abnormal form of haemoglobin (the protein in red blood cells that carries oxygen). The red blood cells become rigid and sticky and change from being disc-shaped to being crescent-shaped (like a sickle).

More about the procedure

The review of Oxbryta was initiated on 29 July 2024 at the request of the European Commission, under Article 20 of Regulation (EC) No 726/2004.

The review has been carried out by the Committee for Medicinal Products for Human Use (CHMP), responsible for questions concerning medicines for human use, which has adopted the Agency's opinion.

The CHMP opinion will now be forwarded to the European Commission, which will issue a final legally binding decision applicable in all EU Member States.

[3] https://www.ema.europa.eu/en/documents/referral/oxbryta-article-20-procedure-suspension-sickle-cell-disease-medicine-oxbryta_en.pdf