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Suspension of sickle cell disease medicine Oxbryta

Measure taken as precaution while review of emerging data is ongoing

On 26 September 2024 EMA's human medicines committee (CHMP) recommended suspending the marketing authorisation for the sickle cell disease medicine Oxbryta (voxelotor); this measure has been taken as a precaution while a review of emerging data is ongoing.

The recommendation followed emerging safety data from two registry-based studies, which indicated that patients in the studies had a higher occurrence of vaso-occlusive crises (VOC) during treatment with Oxbryta than they did before starting the medicine. Vaso-occlusive crises are among the most common complications of sickle cell disease; they involve episodes of acute pain and can lead to further health complications, such as arthritis, kidney failure and stroke.

These new safety data emerged while EMA was already reviewing the benefits and risks of Oxbryta as part of an ongoing review that started in July 2024. This was triggered as data from a clinical trial showed that a higher number of deaths occurred with Oxbryta than with placebo (dummy treatment) and another trial showed the total number of deaths was higher than anticipated.

In this context, the CHMP considered that, overall, these data raise serious concerns about the safety of Oxbryta; due to the increased uncertainties it therefore recommended that the authorisation, marketing and supply of the medicine be suspended until all the available data have been assessed in the ongoing review.

In parallel, the company marketing Oxbryta decided to withdraw and recall the medicine from all countries where it is available, and to discontinue ongoing clinical trials, compassionate use and early access programmes.

While the review is ongoing, EMA recommends that:

- doctors should not start new patients on Oxbryta;
- doctors should contact patients currently treated with Oxbryta to stop treatment and discuss alternative treatment options;
- doctors should continue to monitor patients for adverse events after treatment with Oxbryta is stopped;
- patients must talk to their doctor before stopping their medicine;
- patients who have any questions should talk to their doctor.

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More detailed recommendations will be sent to healthcare professionals prescribing, dispensing or administering the medicine in a direct healthcare professional communication (DHPC). The DHPC will also be published on the EMA website.

EMA's recommendation for a suspension was forwarded to the European Commission, which issued a legally binding decision applicable in all EU Member States on 4 October 2024.

EMA will continue its review of Oxbryta and issue a final recommendation in due course.

More about the medicine

Oxbryta is a medicine used to treat haemolytic anaemia (excess breakdown of red blood cells) in patients aged 12 years and older who have sickle cell disease. Oxbryta can be given on its own or together with another medicine for sickle cell disease called hydroxycarbamide. It 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).

Oxbryta received a marketing authorisation valid throughout the EU on 14 February 2022.

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 is being carried out by the Committee for Medicinal Products for Human Use (CHMP), responsible for questions concerning medicines for human use, which will adopt the Agency's opinion.

The CHMP's recommendation to suspend Oxbryta while the review was ongoing was sent to the European Commission, which issued a legally binding decision on 4 October 2024.