29 July 2024
EMA/450943/2024

Update as of 26 September 2024

EMA's human medicines committee (CHMP) has recommended suspending the marketing authorisation for Oxbryta; this measure is taken as a precaution while emerging data are being assessed in the ongoing review. Further information can be found at the following link.

EMA starts review of sickle cell disease medicine Oxbryta

Review follows reports of fatal cases in clinical trials

EMA has started a review of Oxbryta (voxelotor) after data from a clinical trial showed that a higher number of deaths occurred with Oxbryta than with placebo (a dummy treatment) and another trial showed the total number of deaths was higher than anticipated.

Oxbryta is used to treat haemolytic anaemia (excessive breakdown of red blood cells) due to sickle cell disease in patients from 12 years of age.

One of the studies was assessing the effect of Oxbryta in people with sickle cell disease who were at higher risk of stroke; the other study was evaluating the effect of the medicine on leg ulcers, a known complication of sickle cell disease.

The marketing authorisation holder has now stopped treatment with Oxbryta in both studies while investigations are underway.

There is currently no clear evidence that Oxbryta caused any of the deaths, and information about many of the cases is still being awaited. Some of the cases may have been linked to infections, including malaria.

EMA will now assess information from these studies, taking into account all the available data on the benefits and risks of the medicine. The Agency will then issue a recommendation on whether the marketing authorisation in the EU should be amended, suspended or revoked.

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

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 studies

Study GBT440-032 is assessing the effects of voxelotor on the transcranial doppler ultrasound measurements of cerebral arterial blood flow in children from 2 to 15 years of age who have sickle cell disease and are at high risk of stroke. The study recruited 236 patients from Egypt, Ghana, Kenya, Nigeria, Oman, Saudi Arabia, the United States and the United Kingdom. There were 8 deaths in people taking voxelotor and 2 deaths in people taking placebo.

Study GBT440-042 is assessing the effects of voxelotor on leg ulcers in 88 patients from 12 years of age recruited from Brazil, Kenya and Nigeria. Eight deaths have occurred in the open-label part of this study.

Treatment with voxelotor has been paused for patients in both studies, as well as those who enrolled in a follow-up study (GBT440-038).

There is at present no clear evidence that Oxbryta caused any of the deaths in the trials, and information about many of the cases is still being awaited.

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