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# Oxbryta (*voxelotor*)

An overview of Oxbryta and why it is authorised in the EU

## What is Oxbryta and what is it used for?

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.

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). Sickle cell disease is rare, and Oxbryta was designated an 'orphan medicine' (a medicine used in rare diseases) on 18 November 2016. Further information on the orphan designation can be found here: ema.europa.eu/medicines/human/orphan-designations/eu-3-16-1769.

Oxbryta contains the active substance voxelotor.

## How is Oxbryta used?

Oxbryta can only be obtained with a prescription and treatment should be started by a doctor experienced in the treatment of sickle cell disease.

The medicine is available as tablets to be taken by mouth and the usual recommended dose is 1,500 mg taken once daily.

For more information about using Oxbryta, see the package leaflet or contact your doctor or pharmacist.

## How does Oxbryta work?

In sickle cell disease, sickling of the red blood cells occurs when the abnormal haemoglobin releases its oxygen and then clumps together to form rigid chains that make the cells change shape and cause them to break down more quickly. As well as potentially blocking blood vessels, this leads to lower numbers of red blood cells and less working haemoglobin able to carry oxygen around the body. The active substance in Oxbryta, voxelotor, works by improving the ability of the haemoglobin to hold on to oxygen, and preventing it from forming chains. This helps the red blood cells to maintain normal shape and flexibility, reducing their excess breakdown and improving their lifespan.

**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

## What benefits of Oxbryta have been shown in studies?

Oxbryta has been shown to improve haemolytic anaemia in a main study involving 247 patients aged 12 to 64 years with sickle cell disease. Patients were given Oxbryta or placebo (a dummy treatment) as well as continuing hydroxycarbamide treatment if they were already receiving it. At the start of treatment, the average haemoglobin level was 8.5 g per dL of blood. After treatment for 24 weeks, the haemoglobin level had improved by at least 1 g per dL in around 51% of the group given Oxbryta 1,500 mg daily (46 of 90 patients) versus 6.5% of those taking placebo (6 of 92). Other measures also showed reductions in red blood cell breakdown with Oxbryta.

## What are the risks associated with Oxbryta?

The most common side effects with Oxbryta (which may affect more than 1 in 10 people) are headache, diarrhoea and abdominal (belly) pain. The most serious side effects, affecting around 1 in 100 people, include headache and hypersensitivity (allergic) reactions.

For the full list of side effects and restrictions with Oxbryta, see the package leaflet.

## Why is Oxbryta authorised in the EU?

Current treatments for sickle cell disease already include medicines to prevent the painful crises caused when sickle cells block blood supply to vital organs. However, there is an unmet medical need for treatments to manage the anaemia associated with the condition, which leads to tiredness and chronic pain as well as contributing to other complications. Oxbryta has been shown to improve anaemia by increasing haemoglobin levels and reducing red cell breakdown. It is not yet clear to what extent this will improve clinical symptoms and quality of life in the longer term, since the medicine's action may also reduce the ability of haemoglobin to release oxygen to the body's tissues. Nonetheless, since the side effects seem to be limited and manageable, the European Medicines Agency decided that Oxbryta's benefits are greater than its risks and it can be authorised for use in the EU.

## What measures are being taken to ensure the safe and effective use of Oxbryta?

Recommendations and precautions to be followed by healthcare professionals and patients for the safe and effective use of Oxbryta have been included in the summary of product characteristics and the package leaflet.

As for all medicines, data on the use of Oxbryta are continuously monitored. Suspected side effects reported with Oxbryta are carefully evaluated and any necessary action taken to protect patients.

## Other information about Oxbryta

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

Further information on Oxbryta can be found on the Agency's website: ema.europa.eu/medicines/human/EPAR/oxbryta.

This overview was last updated in 03-2022.