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Xolremdi (*mavorixafor*)

A plain-language overview of Xolremdi and why it is authorised in the EU

What is Xolremdi and what is it used for?

Xolremdi is a medicine for the treatment of WHIM syndrome in adults and adolescents from 12 years of age. It is used to increase the number of circulating mature neutrophils and lymphocytes (types of white blood cells).

WHIM syndrome is an inherited disease in which the immune system (the body's natural defences) does not work properly, making patients more susceptible to bacterial and viral infections. WHIM stands for warts (skin growths, which could potentially become cancerous), hypogammaglobulinaemia (low level of antibodies in the blood), infections and myelokathexis (a condition where certain white blood cells remain trapped in the bone marrow, instead of circulating in the bloodstream, preventing them from fighting infections).

WHIM syndrome is rare, and Xolremdi was designated an 'orphan medicine' (a medicine used in rare diseases) on 25 July 2019. Further information on the orphan designation can be found on the EMA website.

Xolremdi contains the active substance mavorixafor.

How is Xolremdi used?

The medicine can only be obtained with a prescription. Treatment should only be started by specialist doctors with experience in the diagnosis or treatment of immune deficiencies (when the immune system does not function properly).

Xolremdi is available as capsules to be taken by mouth once a day on an empty stomach after an overnight fast, and at least 30 minutes before food.

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

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How does Xolremdi work?

In people with WHIM syndrome, mutations (changes) in the gene for the CXCR4 receptor make it more responsive to CXCL12 (a signal molecule); this increased responsiveness causes mature neutrophils and lymphocytes to remain trapped inside the bone marrow, preventing them from entering the blood to fight infections.

The active substance in Xolremdi, mavorixafor, attaches to and blocks the CXCR4 receptor, preventing its interaction with CXCL12. In this way, mavorixafor helps the release of mature neutrophils and lymphocytes from the bone marrow into the bloodstream, thereby increasing their availability to support the immune system.

What benefits of Xolremdi have been shown in studies?

Xolremdi was shown to be effective at increasing the number of circulating mature neutrophils and lymphocytes in people with WHIM syndrome.

In one main study involving 31 people from 12 years of age with WHIM syndrome, Xolremdi was compared with placebo (a dummy treatment); the main measure of effectiveness was the time patients' neutrophil or lymphocyte counts stayed above a certain level over a 24-hour period, measured four times (every three months) during the one-year study.

In people taking Xolremdi, neutrophil count stayed above 500 cells/microliter on average for 15.0 hours, compared with 2.8 hours in those taking placebo. The time lymphocyte count stayed above 1,000 cells/microliter was 15.8 hours for people taking Xolremdi, compared with 4.6 hours for those in the placebo group. In addition, patients who received Xolremdi had fewer infections than those who received placebo; however, an effect on warts (and therefore on the risk of developing cancer) was not demonstrated.

Studies carried out with Xolremdi are described in more detail in the medicine's assessment report.

What are the side effects and restrictions with Xolremdi?

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

The most common side effects with Xolremdi (which may affect more than 1 in 10 people) include gastrointestinal effects (such as nausea (feeling sick), diarrhoea, vomiting, dyspepsia (indigestion) and abdominal (belly) pain), rash and headache. Gastrointestinal side effects usually resolve within the first 3 months, even if Xolremdi is continued.

Xolremdi must not be used during pregnancy. Because Xolremdi inhibits the CYP2D6 enzyme, it must not be used together with medicines that depend on CYP2D6 (such as dextromethorphan, codeine and tramadol) for their elimination from the body.

Why is Xolremdi authorised in the EU?

Xolremdi was shown to be effective at increasing the number of circulating mature neutrophils and lymphocytes in people with WHIM syndrome, which is expected to reduce the risk of infections. Concerning safety, available data suggest that side effects are generally manageable.

The European Medicines Agency therefore decided that Xolremdi's benefits are greater than its risks and that it can be authorised for use in the EU.

Xolremdi has been authorised under 'exceptional circumstances'. This is because it has not been possible to obtain complete information about Xolremdi due to the rarity of the disease. The company

must provide further data on Xolremdi. It must submit the results of a registry-based study on the long-term safety and effectiveness of Xolremdi, and provide yearly updates on any new information concerning the safety and effectiveness of the medicine. Every year, the Agency will review any new information that becomes available.

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

The company that markets Xolremdi will provide educational materials to doctors expected to prescribe Xolremdi to inform them about the risk of harm to an unborn baby if the medicine is taken during pregnancy. There is also a risk of harm for babies born to men treated with Xolremdi.

Male and female patients will be also provided with a card reminding them of the importance of using effective contraceptive methods (birth control) during treatment with Xolremdi and for three weeks after the last dose, and to contact their doctor if pregnancy is suspected.

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

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

Other information about Xolremdi

Xolremdi received a marketing authorisation under exceptional circumstances valid throughout the EU on 27 April 2026.

Further information, including the package leaflet and assessment report, on Xolremdi can be found on the Agency's website: ema.europa.eu/medicines/human/EPAR/xolremdi.

For information about the availability of this medicine in your country, contact your national competent authority.

This overview was last updated in 04-2026.