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Waskyra (*etuvetidigene autotemcel*)

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

What is Waskyra and what is it used for?

Waskyra is a medicine for treating people aged 6 months and older with Wiskott-Aldrich syndrome (WAS) who have a mutation in the WAS gene. It is used to treat patients for whom a haematopoietic stem cell transplantation is appropriate, but for whom no suitable stem cell donor is available.

Wiskott-Aldrich syndrome is caused by abnormalities in the gene that produces the WAS protein, which is found in blood cells and certain cells of the immune system (the body's natural defences). People with Wiskott-Aldrich syndrome bruise and bleed easily because they have too few normal platelets (components that help the blood to clot); they also have frequent infections because they have too few normal immune cells, which could lead to sepsis (when bacteria and their toxins circulate in the blood and start damaging the organs). In addition, there is a higher risk of developing some types of cancer, such as lymphoma.

Wiskott-Aldrich syndrome is rare, and Waskyra was designated an 'orphan medicine' (a medicine used in rare diseases) on 6 June 2012. Further information on the orphan designation can be found on the EMA website.

Waskyra contains the active substance etuvetidigene autotemcel, which consists of genetically modified stem cells taken from the patient's own blood.

How is Waskyra used?

Waskyra can only be obtained with a prescription and must be given in a qualified treatment centre by a doctor trained in giving this medicine and with experience in stem cell transplantation.

Waskyra is made individually for each patient out of stem cells collected from their blood and must only be given to the patient for whom it is made. It is given once by infusion (drip) into a vein. Before Waskyra is given, the patient will receive conditioning (preparatory) treatment to clear their bone marrow of cells.

For more information about how Waskyra is used, see the package leaflet or contact your doctor or pharmacist.

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How does Waskyra work?

Waskyra is made from stem cells (called CD34+ cells) that are collected from the patient's blood. The cells are genetically modified in the laboratory so that they can produce a functional WAS protein. When transplanted back into the patient, the modified cells migrate to the bone marrow where they start making healthy blood and immune cells that produce a functional WAS protein, thereby helping to relieve the symptoms of the disease.

What benefits of Waskyra have been shown in studies?

The benefits of Waskyra were shown in a clinical development program involving a total of 27 patients with Wiskott-Aldrich syndrome. These included 10 children between 1 and 9 years of age in the main study, and 17 patients between 1 and 35 years of age from another clinical study and an expanded access programme (when people with a serious condition for which there is no authorised treatment are given an investigational medicine outside of a clinical study).

Overall, the data showed that Waskyra is effective at reducing the occurrence of severe infections and bleeding episodes.

The average number of severe infections per year decreased from 2.0 events in the 12 months before treatment to 0.12 events in the 2-3 years after treatment with Waskyra. Similarly, the average number of moderate and severe bleeding episodes per year decreased from 2.0 events in the 12 months before treatment to 0.16 events in the 2-3 years following treatment with Waskyra.

What are the risks associated with Waskyra?

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

The most common side effects reported in people treated with Waskyra were due to the procedures and pre-treatments required to receive the medicine, such as the conditioning regimen, and to administration site conditions (infections related to the infusion device, bleeding at the catheter site).

Waskyra must not be given to people who have previously received a haematopoietic stem cell transplant from a donor and still have donor cells in their blood, or those who have previously received haematopoietic stem cell gene therapy (a treatment in which the patient's stem cells are collected and genetically modified and then returned to the patient).

Why is Waskyra authorised in the EU?

People with Wiskott-Aldrich syndrome for whom haematopoietic stem cell transplantation is appropriate but who have no compatible stem cell donor have a reduced life expectancy. At the time of Waskyra's authorisation, there was a need for effective treatments for these patients.

Treatment with Waskyra resulted in a considerable reduction in severe infections and moderate-to-severe bleeding episodes that is maintained for a long time. The safety of the medicine was considered acceptable, with relevant precautions and measures for monitoring described in the product information. There could be a theoretical risk of cancer caused by unintended changes in the genetic material, although no such cases have been seen so far. Measures are in place to monitor for such events through a 15-year long-term study to better characterise the safety of Waskyra.

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

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

The company that markets Waskyra will conduct and provide the result of a study to further assess the long-term safety and effectiveness of the medicine. The company will also provide educational material for healthcare professionals expected to use the medicine with information on its safety, including the potential risk of cancer and the importance of monitoring and long-term follow-up of people treated with Waskyra. Patients and carers will also receive educational material with information on when to contact their doctor in case of concerns or suspected side effects, how to report side effects and the importance of regular and long-term monitoring.

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

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

Other information about Waskyra

Waskyra received a marketing authorisation valid throughout the EU on 9 January 2026.

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

This overview was last updated in 01-2026.