



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Kymriah (*tisagenlecleucel*)

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

What is Kymriah and what is it used for?

Kymriah is a medicine for treating the following types of blood cancer:

- B-cell acute lymphoblastic leukaemia (ALL), in children and young adults up to 25 years of age whose cancer did not respond to previous treatment, has come back two or more times, or has come back after a transplant of stem cells;
- Diffuse large B-cell lymphoma (DLBCL) and follicular lymphoma (FL) in adults whose cancer has come back or did not respond after two or more previous treatments.

Kymriah is a type of advanced therapy medicine called a 'gene therapy product'. This is a type of medicine that works by delivering genes into the body.

The blood cancers that Kymriah is used to treat are rare, and Kymriah was designated an 'orphan medicine' (a medicine used in rare diseases) for B-cell ALL on [29 April 2014](#), DLBCL on [14 October 2016](#) and FL on [19 July 2021](#).

Kymriah contains the active substance tisagenlecleucel (consisting of genetically modified white blood cells).

How is Kymriah used?

Kymriah is prepared using the patient's own white blood cells which are extracted from the blood and genetically modified in the laboratory.

It is given as a single infusion (drip) into a vein and must only be given to the patient whose cells were used to make it. Before having Kymriah, the patient should have a short course of chemotherapy to clear away their white blood cells and just before the infusion, the patient is given paracetamol and an antihistamine medicine to reduce the risk of reactions to the infusion.

A medicine called tocilizumab and emergency equipment must be available in case the patient has a potentially serious side effect called cytokine release syndrome, a potentially life-threatening condition that can cause fever, vomiting, shortness of breath, pain and low blood pressure (see risks section below).

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Patients should be closely monitored for 10 days after treatment for side effects and are advised to stay close to a specialist hospital for at least 4 weeks after treatment.

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

How does Kymriah work?

Kymriah contains the patient's own T cells (a type of white blood cell) that have been modified genetically in the laboratory so that they make a protein called chimeric antigen receptor (CAR). CAR can attach to another protein on the surface of cancer cells called CD19.

When Kymriah is given to the patient, the modified T cells attach to and kill the cancer cells, thereby helping to clear the cancer from the body.

What benefits of Kymriah have been shown in studies?

B-cell ALL

The main study of Kymriah in B-cell ALL involved 92 children and young adults (3–25 years of age) whose cancer had come back after previous treatment or did not respond to treatment. Around 66% of patients had a complete response (which means they had no signs of the cancer left) in the 3 months after treatment. This was better than results seen with the cancer medicines clofarabine, blinatumomab or a combination of clofarabine, cyclophosphamide and etoposide. Twelve months after treatment, the likelihood of survival was 70%.

DLBCL

The main study of Kymriah in DLBCL involved 165 patients who had at least 2 previous treatments and could not have a stem cell transplant. Around 24% of patients had a complete response and 34% had at least a partial response after at least 3 months. These results were comparable to those from studies of patients receiving standard cancer treatments. Twelve months after treatment, the likelihood of survival was 40%. The majority of patients who responded to Kymriah continued to have a response after 19 months.

FL

The main study for FL involved 98 patients whose cancer had come back after previous treatment or did not respond to treatment. Patients had at least 2 previous treatments. Around 69% of patients had a complete response 3 months after treatment.

What are the risks associated with Kymriah?

Serious side effects occur in most patients. The most common serious side effects are cytokine release syndrome and decreases in platelets (components that help the blood to clot), haemoglobin (the protein found in red blood cells that carries oxygen around the body) or white blood cells including neutrophils and lymphocytes.

In addition, serious infections are very common side effects in patients treated for DLBCL and FL.

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

Why is Kymriah authorised in the EU?

B-cell ALL patients have poor outcomes and improvements with Kymriah were better than with other medicines for this condition. In patients with DLBCL treated with Kymriah, the results were similar to other treatments but ongoing study data indicate that the effects last longer. Kymriah was also shown to produce a robust response in patients with FL. Serious side effects occur in most patients, and can include cytokine release syndrome. However these are manageable if the appropriate measures are in place (see below). The European Medicines Agency therefore decided that Kymriah'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 Kymriah?

The company that markets Kymriah must ensure that hospitals where Kymriah is given have appropriate expertise, facilities and training. Tocilizumab must be available in case of cytokine release syndrome. The company must provide educational materials for healthcare professionals and patients about possible side effects, especially cytokine release syndrome.

The company must carry out several studies to obtain more information on Kymriah including its safety and effectiveness in the long term and in B-cell ALL patients under 3 years of age.

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

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

Other information about Kymriah

Kymriah received a marketing authorisation valid throughout the EU on 23 August 2018.

Further information on Kymriah can be found on the Agency's website: ema.europa.eu/Find/medicine/Human_medicines/European_public_assessment_reports.

This overview was last updated in 04-2022.