

Medicine: tisagenlecleucel (brand name: Kymriah®) for B-cell acute lymphoblastic leukaemia

Novartis Pharmaceuticals UK Ltd

What is tisagenlecleucel used for?

Tisagenlecleucel was considered for the treatment of B-cell acute lymphoblastic leukaemia, which is a very rare type of cancer of the white blood cells. Tisagenlecleucel is used to treat children and adults under 25 years old, where the cancer has not responded to treatment (refractory); has come back after a transplant (relapsed); or has come back after treatment for the second time.

How does tisagenlecleucel work?

Tisagenlecleucel is a new innovative type of medicine called CAR-T cell therapy. It uses the patient's own immune cells (T-cells) to destroy the cancer cells. The patient's T-cells are collected and modified in the laboratory to create CAR-T cells. These cells are then given back to the patient through a drip into the vein. The CAR-T cells bind to and kill the cancer cells.

What has SMC said?

SMC has accepted tisagenlecleucel for the treatment of children or adults under the age of 25 years with B-cell acute lymphoblastic leukaemia as described above.



Why has SMC said this?

SMC looks at how well new medicines work compared with current treatments available in Scotland and in relation to how much they will cost to buy and administer (for example, if the medicine has to be given at a clinic or side effects have to be monitored).

SMC carefully considers every new medicine to make sure it benefits patients and is considered to be an acceptable use of the limited resources in NHSScotland. When SMC assesses a medicine it takes account of the needs of all patients in NHSScotland, not just those who may be treated with the medicine under consideration.

To do this SMC consider the following:

- Clinical trial and economic evidence from the company that makes the medicine.

- Advice from healthcare professionals about any benefits of the new medicine compared to current treatment, along with how the new medicine is likely to be used.
- Information from patient groups about the potential impact of the medicine on patients and carers.

After careful consideration, applying extra flexibility because tisagenlecleucel is a medicine for a very rare, end of life condition, and after the company applied a confidential discount to the cost of the medicine, SMC was able to accept it as a possible treatment within NHSScotland.

What does SMC's decision mean for me?

If your healthcare professional thinks that tisagenlecleucel for use as described above is the right medicine for you or your child, the treatment should be available on the NHS. For further information see:

Medicines in Scotland: What's the right treatment for me?

www.healthcareimprovementscotland.org/medicinesbooklet.aspx



More information

The organisations below can provide more information and support for people with leukaemia and their families. SMC is not responsible for the content of any information provided by external organisations.

Bloodwise



<https://www.bloodwise.org.uk>



0808 2080 888

Leukaemia CARE



<https://www.leukaemiacare.org.uk/>



08088 010 444

You can find out more about tisagenlecleucel in the European public assessment report (EPAR) summary for the public by searching for the medicine name on the European Medicines Agency (EMA) website.



<http://www.ema.europa.eu>

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