

Children and young adults with acute type of leukaemia to get continued access to CAR-T treatment following NICE recommendation

Apr 11, 2024

- *The National Institute for Health and Care Excellence (NICE) has recommended Kymriah®▼ (tisagenlecleucel) for treating paediatric and young adults up to and including the age of 25 with B-cell acute lymphoblastic leukaemia (ALL) that is refractory, in relapse post-transplant or in second or later relapse*
- *NICE recommendation clears the way for routine funding following exit from the Cancer Drugs Fund, through which tisagenlecleucel has already treated over 133 patients with ALL¹*
- *ALL causes a range of physical and psychological symptoms that can be particularly acutely felt in children and young adults. The treatment will continue to address unmet need in the disease area amongst eligible patients*
- *The treatment becomes only the second CAR-T cell therapy to secure routine funding for use in the NHS, and the only one for treating ALL in children and young adults under 26 years old.*

London, 11 April 2024 - Today, Novartis announced that the National Institute for Health and Care Excellence (NICE) has issued final draft guidance (MA review of TA554), recommending Kymriah®▼ (tisagenlecleucel) for treating paediatric and young adults up to and including the age of 25 with B-cell acute lymphoblastic leukaemia (ALL) that is refractory, in relapse post-transplant or in second or later relapse.

ALL is a rare type of cancer affecting the blood and bone marrow, caused by the overproduction of lymphoblasts and develops rapidly. The disease is most common among young children, with the B-cell category of ALL accounting for 78% of cases in children.¹

Responding to the recommendation, Charlotte Crowley, Policy and Evidence Manager of Leukaemia Care said: *"Today's decision will ensure ALL patients will continue to access this treatment. These patients have few other options for treatment and CAR-T therapies have revolutionised the treatment of these patients already. We are really pleased that NICE has been able to recommend this treatment for the longer term, giving patients and doctors much greater clarity on what is available if they do find themselves in this position."*

The recommendation was based on longer follow-up clinical data from the ELIANA, ENSIGN,^{3,4} and B2101J^{5,6} trials that were initially used at approval in 2018, as well as new data collected during its use through the Cancer Drugs Fund (CDF).¹

"During its time in the CDF, tisagenlecleucel has changed the way in which people with relapsed or refractory B-ALL have treatment," said Dr Sara Ghorashian from Great Ormond Street Hospital. *"It offers a chance of durable remissions and prolonged overall survival for people who often have no other option. The CDF has enabled us to build robust real-world evidence and I'm delighted that NICE has recommended that children and young adults should continue to have access to this treatment."*

“Novartis have been working with NHS England to maintain access to CAR-T therapy in the areas of highest unmet need since its first approval in 2018,” said Marie-Andrée Gamache, President and Managing Director of Novartis UK and Ireland. “Cell and gene therapies are another example of how we’re reimagining medicine to make a real difference to patients’ lives. While in the Cancer Drugs Fund, tisagenlecleucel has been used to treat 133 children and young adults with a potentially deadly form of cancer and today’s NICE recommendation provides the opportunity for it to continue being a treatment option for many more.”

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About B-cell acute lymphoblastic leukaemia (ALL)

ALL is a rare type of blood cancer that starts from white blood cells called lymphocytes which normally help to fight infections in the body. In ALL, lymphocytes that have not fully developed, known as lymphoblasts, become cancerous and are overproduced, gathering in the bone marrow where new blood cells are made.³

The disease is known as relapsed ALL when people experience a period of time in remission where the disease responds to treatment, but leukaemia cells then reappear, known as a relapse. When the disease does not respond to treatment, the disease is known as refractory ALL.⁹

ALL can be grouped as either B-cell or T-cell ALL, with B-cell ALL being more common in children.¹⁰

About Kymriah®▼ (tisagenlecleucel)

Tisagenlecleucel is an autologous, immunocellular cancer therapy which involves reprogramming a patient’s own T cells with a transgene encoding a chimeric antigen receptor (CAR) to identify and eliminate CD19 expressing cells.¹¹

▼ This medicinal product is subject to additional monitoring. This will allow quick identification of new safety information. Healthcare professionals are asked to report any suspected adverse reactions. Reporting forms and information can be found at www.mhra.gov.uk/yellowcard.

About ELIANA, ENSIGN and B2101J clinical trials

ELIANA was an international, multicentre, phase II trial which included paediatric and young adult patients (aged 3 years at screening to 21 at initial diagnosis) with r/r B-cell ALL. Data collected from ELIANA have been reported in the journal article by Laetsch et al. (2022)¹². This submission used the latest ELIANA data collected in November 2022. By November 2022, 79 patients had received a tisagenlecleucel infusion.⁵

ENSIGN was a US-based, multicentre, phase II, single-arm, open-label study to assess efficacy and safety. It similarly included paediatric and young adult patients (aged 3 years at screening to 21 at initial diagnosis) with r/r B-cell ALL. By May 2019, 64 patients had received a tisagenlecleucel infusion.⁷

B2101J was the first tisagenlecleucel trial and was a US-based, phase I/IIa trial which included paediatric and young adult patients aged up to 24 years with chemotherapy resistant or refractory CD19+ B-cell leukaemia and lymphoma. By May 2018, a total of 57 patients with non-CNS3 ALL had received a tisagenlecleucel infusion.⁹

About Novartis

Novartis is an innovative medicines company. Every day, we work to reimagine medicine to improve and extend people's lives so that patients, healthcare professionals and societies are empowered in the face of serious disease.

In the UK, we champion health and lives through pioneering NHS partnerships, innovative collaborations and a clear focus on the greatest healthcare challenges we all face. We are where science meets hope.

To reimagine medicine with us, visit our website at <https://www.novartis.com/uk-en/> and connect on [LinkedIn](#), [Facebook](#), [X/Twitter](#) and [Instagram](#).

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