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Abecma *(idecabtagene vicleucel)*

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

What is Abecma and what is it used for?

Abecma is a medicine used to treat adults with multiple myeloma (a cancer of the bone marrow) when the cancer has come back (relapsed) and has not responded to treatment (refractory). It is used in adults who have received at least two prior therapies, including an immunomodulatory agent, a proteasome inhibitor and an anti-CD38 antibody and whose disease has worsened since the last treatment.

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

Multiple myeloma is rare, and Abecma was designated an 'orphan medicine' (a medicine used in rare diseases) on 20 April 2017. Further information on the orphan designation can be found here: https://www.ema.europa.eu/en/medicines/human/orphan-designations/eu3171863

Abecma contains the active substance idecabtagene vicleucel, which consists of genetically modified T cells (a type of white blood cells).

How is Abecma used?

Abecma is prepared using the patient's own T cells which are extracted from the blood and genetically modified in the laboratory. Abecma can only be given to the patient whose cells were used to make the medicine.

It is given as a single infusion (drip) into a vein. Before having Abecma, the patient should receive a short course of chemotherapy to clear away their existing white blood cells. Just before the infusion patients are 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 (CRS; a potentially life-threatening overactivation of the immune system with fever, shortness of breath, low blood pressure and headache).

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

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For more information about using Abecma, see the package leaflet or contact your healthcare provider.

How does Abecma work?

Abecma contains the patient's own T cells which have been modified genetically in the laboratory so that they make a protein called chimeric antigen receptor (CAR). CAR can attach on the surface of cancer cells to a protein called B-cell maturation antigen (BCMA).

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

What benefits of Abecma have been shown in studies?

A main study of 140 patients with multiple myeloma that was not responding to at least three previous treatments (refractory myeloma) and had returned (relapsed myeloma) showed that Abecma is effective at clearing the cancer. The study did not compare Abecma with other medicines or placebo (a dummy treatment). Overall 30% of patients had a complete response (which means they had no signs of the cancer left) and 67% had at least a partial response after treatment with Abecma.

An additional study looked at 386 patients with multiple myeloma who had received at least two previous treatments. This study compared Abecma with other medicines to treat multiple myeloma (standard treatments). Patients on Abecma lived on average for 13.8 months without their disease getting worse compared with 4.4 months for those on standard treatments. Overall, 71% of patients responded to treatment with Abecma, compared to 42% of patients on standard treatments. The response lasted on average 16.5 months with Abecma compared with 9.7 months with standard treatments.

What are the risks associated with Abecma?

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

The most common side effects (which may affect more than 1 in 5 people) include neutropenia (low levels of neutrophils, a type of white blood cell), CRS, anaemia (low levels of red blood cells), thrombocytopenia (low levels of platelets, components that help the blood to clot), infections, hypophosphataemia (low blood phosphate levels), diarrhoea, leucopenia (low levels of white blood cells), hypokalaemia (low blood potassium levels), tiredness, nausea (feeling sick), lymphopenia (low levels of lymphocytes), fever, viral infections, headache, hypocalcaemia (low blood levels of calcium), hypomagnesaemia (low blood levels of magnesium), and joint pain.

The most common serious side effects include CRS (10%) and pneumonia (infection of the lungs; 7%).

People who cannot have chemotherapy to clear away their existing white blood cells must not receive Abecma.

Why is Abecma authorised in the EU?

Abecma led to clinically meaningful response rates in patients with multiple myeloma when the cancer had relapsed and not responded to treatment. Patients on Abecma also lived for longer without their disease getting worse compared with standard treatment. Serious side effects, particularly CRS, can occur; however, these are manageable if appropriate measures are in place (see below). The European Medicines Agency decided that Abecma's benefits are greater than its risks and it can be authorised for use in the EU.

Abecma was originally given 'conditional authorisation' because there was more evidence to come about the medicine. As the company has supplied the additional information necessary, the authorisation has been switched from conditional to standard authorisation.

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

The company that markets Abecma must ensure that hospitals where the medicine is given have appropriate expertise, facilities and training. Tocilizumab must be available in case of CRS. In addition the company will provide educational programme on how to identify and manage CRS for healthcare professionals involved in the treatment of patients . The company must also provide patient cards with information about CRS and serious side effects affecting the nervous system, and when and where to seek help if these occur. This card will also inform healthcare professionals that the patient is receiving Abecma.

The company must also carry out a study to obtain more information on the long-term safety and effectiveness of Abecma.

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

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

Other information about Abecma

Abecma received a conditional marketing authorisation valid throughout the EU on 18 August 2021. This was switched to a standard marketing authorisation on 19 March 2024.

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

This overview was last updated in 03-2024.