EMA/614446/2021
EMEA/H/C/00462

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 three 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.

How is Abecma used?

Abecma is prepared using the patient's own white blood 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 (see risks section below).

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.

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

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How does Abecma work?

Abecma 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 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 previous treatment (refractory myeloma) and had returned (relapsed myeloma) showed that Abecma is effective at clearing the cancer. Overall 30% 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.

What are the risks associated with Abecma?

The most common side effects (which may affect more than 1 in 10 people) are neutropenia (low levels of neutrophils, a type of white blood cell), cytokine release syndrome (a potentially life-threatening condition that can cause fever, vomiting, shortness of breath, pain and low blood pressure), anaemia (low red blood cell count), thrombocytopenia (low platelet counts), infections, leucopenia (low white blood cell count), tiredness, diarrhoea, hypokalaemia (low blood potassium levels), hypophosphataemia (low blood phosphate levels), nausea (feeling sick), lymphopenia (low levels of lymphocytes), fever, cough, hypocalcaemia (low blood levels of calcium), headache, hypomagnesaemia (low blood levels of magnesium), upper respiratory tract infection (nose and throat infection), joint pain, peripheral oedema (swelling especially of the ankles and feet), decreased appetite, hypogammaglobulinaemia (low levels of immunoglobulins) and febrile neutropenia (low blood levels of neutrophils with fever).

The most common serious side effects include cytokine release syndrome (17%), pneumonia (7%), febrile neutropenia (6%) and fever (6%).

People who cannot have chemotherapy to clear away their existing white blood cells (as per the package leaflet of the relevant chemotherapy) must not receive Abecma.

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

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. Serious side effects, particularly cytokine release syndrome, 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 has been given 'conditional authorisation'. This means that there is more evidence to come about the medicine, which the company is required to provide. Every year, the Agency will review any new information that becomes available and this overview will be updated as necessary.

What information is still awaited for Abecma?

Since Abecma has been given conditional authorisation, the company that markets Abecma will provide 24 months follow-up data on patients from the main study. In addition the company will carry out a study to compare Abecma with standard chemotherapy in people with relapsed and refractory multiple myeloma.

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 cytokine release syndrome.
- provide educational materials for healthcare professionals and patients about possible side effects, especially cytokine release syndrome.

The company must also carry out a study to obtain more information on the long term safety 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

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