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Zemcelpro (*dorocubicel / unexpanded CD34- cells*)

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

What is Zemcelpro and what is it used for?

Zemcelpro is a stem-cell medicine used to treat adults with haematological malignancies (blood cell cancers) who need an allogeneic haematopoietic stem cell transplantation (allo-HSCT) and for whom no other type of suitable donor cells is available.

Allo-HSCT is a procedure where the patient's bone marrow (spongy tissue inside the large bones where blood cells are produced) is cleared of cells, which are then replaced by cells from a matched donor; the transplanted cells multiply and develop into healthy specialised blood and immune cells. Zemcelpro is given after patients have received conditioning (preparatory) treatment with cancer medicines to clear cells from the bone marrow.

HSCT is a rare procedure, and Zemcelpro was designated an 'orphan medicine' (a medicine used in rare diseases) for use in patients needing an HSCT on 22 April 2020. Further information on the orphan designation can be found on the EMA website.

Zemcelpro is made specifically for each patient. It contains two types of stem cells from donated umbilical cord blood: dorocubicel (expanded CD34+ cells; expanded means that the cells have been grown and multiplied in the laboratory) and unexpanded CD34- cells.

How is Zemcelpro used?

Zemcelpro can only be obtained with a prescription. It must be administered in a qualified transplant centre with expertise in HSCT, by a doctor with experience in the treatment of blood cell cancers.

Zemcelpro is given once as a single dose, by infusion (drip) into a vein. A single dose of Zemcelpro consists of 1 to 4 infusion bags containing dorocubicel and 4 bags containing unexpanded CD34- cells.

Before and after treatment with Zemcelpro, patients are given other medicines to reduce the risk of reactions linked to the infusion and to prevent transplant complications.

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

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How does Zemcelpro work?

Zemcelpro is used to treat patients with blood cancers who need an allo-HSCT and for whom no other type of suitable donor cells is available.

Umbilical cord blood cells can be used in these patients; however, for some patients no suitable umbilical cord blood is available due to a low number of stem cells in the donated umbilical cord blood. A low number of stem cells in umbilical cord blood can delay engraftment (when the transplanted cells start growing and producing healthy blood cells).

In Zemcelpro, some of the umbilical cord stem cells are grown and multiplied in the laboratory and are then given along with unexpanded cells from the same umbilical cord blood. The expanded CD34+ cells mainly promote growth of healthy blood cells, while the unexpanded CD34- cells support this and also help to remove any remaining cancer cells. After the infusion, the stem cells from Zemcelpro migrate to the bone marrow where they multiply, mature and develop into healthy specialised blood and immune cells.

What benefits of Zemcelpro have been shown in studies?

The benefits of Zemcelpro were shown in two main studies including 25 patients with high-risk leukaemia or myelodysplasia (types of blood cancer) who needed an allo-HSCT and for whom no other type of suitable donor cells was available. In the studies, which are ongoing, Zemcelpro was not compared with any other treatment.

The studies showed that the average time it took for patients to achieve neutrophil engraftment (when the transplanted cells start growing and producing neutrophils, a type of white blood cell) after receiving Zemcelpro was 20 days. By day 42, 21 out of 25 patients (84%) had achieved neutrophil engraftment.

In addition, patients achieved platelet engraftment (when the transplanted cells start growing and producing platelets, components that help the blood to clot) after an average of 40 days after receiving Zemcelpro; by day 100, 17 out of 25 patients (68%) had achieved platelet engraftment.

The above results indicate that the transplant was successful and that new blood cells were starting to be produced in the bone marrow.

What are the risks associated with Zemcelpro?

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

The most common side effects with Zemcelpro (which may affect more than 1 in 10 people) include lymphopenia (low levels of lymphocytes, a type of white blood cell), infections, anaemia (low levels of red blood cells), neutropenia (low levels of neutrophils), thrombocytopenia (low levels of blood platelets), leucopenia (low levels of white blood cells), hypogammaglobulinaemia (reduced blood levels of antibodies), febrile neutropenia (low levels of neutrophils with fever), hypertension (high blood pressure), engraftment syndrome (a complication of HSCT with symptoms such as fever, rash and other inflammatory responses) and pneumonia (lung infection). Acute graft-versus-host disease (GvHD, when donor cells attack the body shortly after a transplant) was reported in 60% of patients, and chronic GvHD (which usually develops later than acute GvHD, within several weeks to months after a transplant) was reported in 16% of patients.

Side effects resulting in death occurred in around 8% of patients treated with Zemcelpro, and included infections, acute GvHD, pulmonary alveolar haemorrhage (a condition where bleeding occurs into the air sacs of the lungs), pneumonitis (inflammation in the lungs, such as idiopathic pneumonia syndrome

and cryptogenic organising pneumonia), and pulmonary hypertension (high blood pressure in the blood vessels that supply the lungs).

Why is Zemcelpro authorised in the EU?

At the time of authorisation, the treatment of patients with blood cell cancers who needed an HSCT and for whom no other type of suitable donor cells was available represented a clinical challenge. These patients had no other treatment options available and a minimal chance of recovery.

Based on the results from a small number of patients in the ongoing studies, the recovery of neutrophils and platelets after treatment with Zemcelpro was rapid, robust and sustained, indicating that Zemcelpro can be of benefit to these patients. The safety of Zemcelpro was considered acceptable in these patients with no other treatment options at the time of authorisation.

Zemcelpro has been given conditional authorisation for use in the EU. This means that it has been authorised on the basis of less comprehensive data than are normally required because it fulfils an unmet medical need. The European Medicines Agency considers that the benefit of having the medicine available earlier outweighs any risks associated with using it while awaiting further evidence.

The company must provide further data on Zemcelpro. It must submit the final results of the two ongoing main studies and of two additional studies with Zemcelpro and provide data from another study based on a registry of patients treated with Zemcelpro. Every year, the Agency will review any new information that becomes available.

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

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

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

Other information about Zemcelpro

Zemcelpro received a conditional marketing authorisation valid throughout the EU on 25 August 2025.

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

This overview was last updated in 08-2025.