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New stem cell therapy to treat patients with blood cancers

20 June 2025

Zemcelpro provides an option for patients with blood cancer who need a blood stem cell transplant and have no suitable donor

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EMA has recommended granting a conditional marketing authorisation in the European Union (EU) for Zemcelpro (dorocubicel / unexpanded umbilical cord cells) to treat adults with haematological malignancies (blood cell cancers). Zemcelpro can be used in patients requiring an allogeneic haematopoietic stem cell transplantation (allo-HSCT, transplantation of stems cell from a donor) following myeloablative conditioning (chemotherapy and/or radiotherapy) for whom no other type of suitable donor cells is available.

Haematological malignancies are blood cell cancers categorised depending on where they are first detected and include leukaemias (blood), lymphomas (lymph nodes), myelodysplastic syndrome and myelomas (bone marrow). They are frequently diagnosed cancers, and the only potential curative treatment option for several of these cancers is allo-HSCT. This type of transplant involves using donated stem cells to replace the recipient’s bone marrow cells to form new bone marrow that produces healthy blood cells.

Stem cells used for transplantation are preferentially sourced from a matched donor, including a matched sibling or a matched unrelated donor. Umbilical cord blood cells can be used in patients who lack access to any type of suitable donor. However, the number of stem cells in umbilical cord blood is often low and can delay engraftment, the successful establishment and proliferation of the donor stem cells in the recipient's bone marrow.

Zemcelpro is a cell therapy containing stem cells from a donor’s umbilical cord blood, some of which have been grown and multiplied (dorocubicel). By increasing the number of cells, Zemcelpro makes the stem cells from a small cord blood unit more effective.

Zemcelpro was supported through EMA's PRIority MEdicines (PRIME) scheme, which provides early and enhanced scientific and regulatory support to medicines that have a particular potential to address patients' unmet medical needs.

The recommendation is largely based on a pooled analysis of two single arm, open-label studies which included 25 patients. In total, 21/25 (84%) patients achieved neutrophil engraftment (when donor stem cells successfully establish themselves in the recipient's bone marrow and produce neutrophils, a type of white blood cell) within a median time of 20 days, and 17 (68%) patients achieved platelet engraftment within a median time of 40 days.

The most common side effects observed in a wider pool of 116 patients treated with Zemcelpro include low levels of various types of blood cells and of antibodies that help fight infections, high blood pressure, infections, and engraftment syndrome, an inflammatory condition that can occur after HSCT. Acute graft-versus-host disease (GvHD), when donor/transplanted cells attack the body shortly after a transplant) up to 100 days after transplantation was reported in 60% of patients, and chronic GvHD appearing up to one year after transplantation was reported in 13% of patients. Monitoring and mitigation strategies for these side effects are described in the product information and in the risk management plan.

In its overall assessment of the available data, the Committee for Advanced Therapies (CAT), EMA's expert committee for cell-

and gene-based medicines, found that the benefits of Zemcelpro outweighed the possible risks in patients with haematological malignancies requiring allo-HSCT for whom no matched donor cells are available. The CHMP, EMA's human medicines committee, agreed with the CAT's assessment and positive opinion, and recommended approval of this medicine.

Zemcelpro is recommended for a conditional marketing authorisation, one of the EU's regulatory mechanisms to facilitate early access to medicines that fulfil an unmet medical need. This type of approval allows the Agency to recommend a medicine for marketing authorisation with less complete data than normally expected, if the benefit of a medicine's immediate availability to patients outweighs the risk inherent in the fact that not all the data are yet available.

In order to confirm the safety and efficacy of Zemcelpro, the company has been requested to submit long-term follow-up results of the single arm studies, conduct a randomised controlled study and a study based on a patient registry.

The opinion adopted by the CHMP is an intermediate step on Zemcelpro's path to patient access. The opinion will now be sent to the European Commission for the adoption of a decision on an EU-wide marketing authorisation. Once a marketing authorisation has been granted, decisions about price and reimbursement will take place at the level of each Member State, taking into account the potential role or use of this medicine in the context of the national health system of that country.

Notes:

- The applicant for Zemcelpro is Cordex Biologics International Limited.
- Zemcelpro was granted eligibility to PRIME on 10 December 2020 for urgent allogeneic haematopoietic stem cell transplantations.
- Zemcelpro was designated as an orphan medicinal product on 22 April 2020 for treatment in hematopoietic stem cell transplantation. Following this positive CHMP opinion, the Committee for Orphan Medicinal Products (COMP) will assess whether the orphan designation should be

maintained.

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