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Withdrawal of application for the marketing authorisation of Luxceptar (viable T-cells)

Kiadis Pharma Netherlands B.V. withdrew its application for a marketing authorisation of Luxceptar for the treatment of patients with blood cancers who are receiving a type of blood stem cell transplant.

The company withdrew the application on 6 November 2019.

What is Luxceptar and what was it intended to be used for?

Luxceptar is a medicine developed to improve survival in patients with blood cancers being treated with a specific type of blood stem cell transplant (a procedure to replace a patient's unhealthy blood-forming cells with healthy ones from a matched donor).

Luxceptar was expected to be used along with the transplant in patients with certain blood cell cancers that have responded to chemotherapy and radiation treatment (complete remission) but are at high risk of coming back.

It was to be used in patients receiving a transplant from a related but not identical donor and from which T-cells (a type of white blood cell) have been removed. T-cells from such a transplant can attack healthy cells in their new host and cause serious side effects called graft-versus-host disease (GVHD); removing them reduces this risk but reduces the likelihood that the transplant will be effective.

Luxceptar contains T-cells from the transplant donor, specially treated to reduce the risk of GVHD. It was to be available as a liquid suspension to be given by infusion (drip) into a vein.

Luxceptar was designated an 'orphan medicine' (a medicine used in rare diseases) on various dates. Further information on the orphan designations can be found on the Agency's website: prevention of GVHD, September 2008; treatment of acute myeloid leukaemia, November 2014; treatment in haematopoietic stem cell transplantation, June 2016.

How does Luxceptar work?

Although reducing the number of transplanted T-cells in a transplant reduces the risk of GVHD, it can also make it harder for the transplant to fight against the cancer cells and rebuild the patient's immune

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

system. Luxceptar contains the donor's T-cells that have been selectively treated just to remove those that might attack the patient's tissue and cause GVHD. Giving them shortly after the transplant replaces the missing cells in the transplant and helps it to fight infections and cancer cells.

What did the company present to support its application?

The company provided results from a main study involving 23 patients who were to receive a T-cell-depleted blood stem cell transplant from a related but not identical donor to treat acute myeloid or lymphoblastic leukaemias or myelodysplastic syndromes. The main measure of effectiveness was the number of deaths related to transplant complications after 6 months. The company also provided supporting data on quality, safety and effectiveness from other studies.

How far into the evaluation was the application when it was withdrawn?

The application was withdrawn after the European Medicines Agency had evaluated the information from the company and prepared questions for the company. After the Agency had assessed the company's responses to the last round of questions, and had subsequently consulted external experts and the company, there were still some unresolved issues.

Because Luxceptar is an advanced therapy medicine, it was assessed on the Agency's behalf by the Committee for Advanced Therapies.

What did the Agency recommend at that time?

Based on the review of the data and the company's responses to the Agency's questions, at the time of the withdrawal, the Agency had some concerns and its provisional opinion was that Luxceptar could not have been authorised for treating patients with blood cancers receiving T-cell depleted blood stem cell transplants. The main study involved a small number of patients and the way it was designed did not allow the Agency to reach a conclusion on the effectiveness of Luxceptar. Therefore, at the time of the withdrawal, the Agency's opinion was that, because effectiveness was not proven, the benefits of Luxceptar did not outweigh its risks.

What were the reasons given by the company for withdrawing the application?

In its letter notifying the Agency of the withdrawal of the application, the company stated that it was withdrawing the application because of the Agency's view that the current evidence was not sufficient to conclude on the effectiveness of treatment.

Does this withdrawal affect patients in clinical trials?

The company informed the Agency that there are no consequences for patients in clinical trials using Luxceptar.

If you are in a clinical trial and need more information about your treatment, speak with your clinical trial doctor.