



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Outcome of assessment on use of Vyxeos liposomal in the treatment of relapsed or refractory acute myeloid leukaemia

The European Medicines Agency has finalised its assessment of an application to extend the use of Vyxeos liposomal to include the treatment of young patients (aged 1 to 21 years) with acute myeloid leukaemia (AML) that has come back (relapsed) or has not responded (refractory) to previous treatment.

Although EMA did not recommend this use, it agreed that relevant data from the study submitted with the application is to be included in the medicine's product information so that healthcare professionals have access to up-to-date data on the effects of Vyxeos liposomal in young patients (aged 1 to 21 years) with relapsing or refractory AML.

What is Vyxeos liposomal and what is it used for?

Vyxeos liposomal is a cancer medicine used to treat adults with newly diagnosed AML, a cancer of white blood cells. It is used when the leukaemia was caused by previous treatments (e.g. for other cancers) or is associated with certain changes in the bone marrow known as myelodysplasia.

The active substances in Vyxeos liposomal are daunorubicin and cytarabine. It is available as an infusion (drip) into a vein.

Vyxeos liposomal has been authorised in the EU since August 2018.

Further information on Vyxeos liposomal's current uses can be found on the Agency's website:

<https://www.ema.europa.eu/en/medicines/human/EPAR/vyxeos-liposomal>

What change had the company applied for?

The company applied to extend the use of Vyxeos liposomal to the treatment of relapsed or refractory AML in young patients (aged 1 to 21 years).

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How does Vyxeos liposomal work?

The active substances in Vyxeos liposomal, daunorubicin and cytarabine, have been used together to treat leukaemia and other types of cancer for many years. They interfere in different ways with the production of new DNA within cells, which means the cells are unable to grow and multiply, and they eventually die.

In this medicine, daunorubicin and cytarabine are contained in tiny fat droplets called 'liposomes'. The liposomes are expected to remain in the patient's body for longer than conventional daunorubicin and cytarabine medicines and to build up in the patient's bone marrow. The liposomes protect the cancer medicines from being broken down early, which is expected to enhance their effect on cancer cells.

What did the company present to support its application?

To support its application, the company provided data from a study involving 38 children and adolescents with relapsed AML who received Vyxeos liposomal. Patients' remission of AML was analysed after treatment with Vyxeos liposomal.

What were EMA's conclusions?

EMA noted several limitations in the data presented from the main study. While the Agency acknowledged that the main measure of effectiveness in the study (remission of AML) is important, it was unclear what the long-term benefit in patients with relapsed AML would be. Also, patients were not analysed by age group, of which some could have had different treatment options in addition to Vyxeos liposomal.

The company decided to withdraw the applied new indication without addressing the issues identified by EMA. Vyxeos liposomal will therefore not be authorised for relapsed or refractory AML in patients aged 1 to 21 years old. The prescribing information for Vyxeos liposomal will be updated to include relevant data, so that healthcare professionals have access to up-to-date data on the effects of Vyxeos liposomal in young patients (aged 1 to 21 years) with AML.

Does this outcome affect patients in clinical trials / compassionate use programmes?

The company informed the Agency that there are no consequences for patients in clinical trials or in compassionate use programmes using Vyxeos liposomal.

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