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Refusal of the marketing authorisation for Vanflyta (quizartinib)

The European Medicines Agency has recommended the refusal of the marketing authorisation for Vanflyta, a medicine intended for the treatment of adults with acute myeloid leukaemia (AML) (a cancer of the white blood cells).

The Agency issued its opinion on 17 October 2019. The company that applied for authorisation, Daiichi Sankyo Europe GmbH, may ask for re-examination of the opinion within 15 days of receiving the opinion.

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

Vanflyta was developed as a cancer medicine for the treatment of a type of AML called 'FLT3-ITD positive' (when the cancer cells have a certain change in the gene for a protein called FLT3). Vanflyta was to be used in adult patients whose disease had come back or did not respond to previous treatments and for continuation of treatment after the patient had undergone a haematopoietic stem cell transplant (HSCT, a transplant of cells that can develop into different types of blood cells).

Vanflyta contains the active substance quizartinib and was to be available as tablets.

Vanflyta was designated an 'orphan medicine' (a medicine used in rare diseases) on 23 March 2009 for the treatment of AML. Further information on the orphan designation can be found on the Agency's website: ema.europa.eu/medicines/human/orphan-designations/eu309622.

How does Vanflyta work?

The active substance in Vanflyta, quizartinib, is a 'receptor tyrosine kinase inhibitor'. It works by blocking FLT3, a protein involved in cell growth and proliferation. By blocking FLT3, quizartinib is expected to stop cancer cells from multiplying and thus slow down the progression of the disease.

What did the company present to support its application?

The company presented the results of a study in 367 patients with FLT3-ITD positive AML whose disease did not respond to treatment or came back after treatment. Vanflyta was compared with other

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
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cancer medicines and the main measure of effectiveness was overall survival (how long patients lived) after being given Vanflyta or the comparator medicines.

What were the main reasons for refusing the marketing authorisation?

Although the results from the main study indicated a marginal improvement in overall survival for patients given Vanflyta, the study had important limitations which meant that the effectiveness of Vanflyta could not be sufficiently demonstrated.

Therefore, the Agency's opinion was that the benefits of Vanflyta did not outweigh its risks and it recommended refusing marketing authorisation.

Does this refusal affect patients in clinical trials or compassionate use programmes?

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

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