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Precautionary marketing suspension of thalassaemia medicine Zynteglo

The company that markets the gene therapy medicine Zynteglo for treating the rare blood condition beta thalassaemia has suspended sales pending investigation of a safety concern.

The company, bluebird bio, notified EMA that a related medicine it was developing, which uses the same technology as Zynteglo, may have been associated with a case of cancer. Although no cases of cancer have been reported with Zynteglo itself, the company suspended marketing of Zynteglo until the possibility that the same risk might apply to the licensed medicine has been investigated.

The concern arose with the medicine, bb1111, intended to treat another blood disorder, sickle cell disease. This medicine uses the same viral vector as Zynteglo, based on a type of virus known as a lentivirus, to insert a working gene into the patient's blood cells. One patient treated with bb1111 developed acute myeloid leukaemia, a cancer of the blood, that might have been related to treatment, and a different blood disorder, myelodysplastic syndrome, was reported in another patient.

Cancer caused by this type of treatment (insertional oncogenesis) was already identified as a potential risk with Zynteglo, so patients who receive the medicine are followed up and monitored in a registry. So far no cases of cancer have been reported with Zynteglo treatment. Nonetheless, since bb1111 works in the same way, it was thought prudent to suspend clinical studies with bb1111 and pause sales of Zynteglo while the evidence is examined more thoroughly.

EMA is liaising closely with the company and experts within the regulatory network, and will now examine the evidence at EU level and decide on any relevant regulatory action for Zynteglo or any similar medicines under evaluation. No other authorised medicines use the same viral vector so no direct implications are foreseen for other licensed medicines.

Zynteglo was granted conditional marketing authorisation on 29 May 2019. Currently it is only marketed in Germany, and because of limited availability and the rarity of the condition it is intended to treat, only a very small number of patients have received or would have been eligible to receive treatment. However, if treated patients do have any concerns they should contact the specialist supervising their Zynteglo treatment.

EMA will communicate further once additional information becomes available.

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More about the medicine

Patients with beta-thalassaemia cannot make enough beta-globin, a component of haemoglobin, the protein in red blood cells that carries oxygen around the body. As a result, these patients have low red blood cell levels and need frequent blood transfusions. Zynteglo is made by taking stem cells from the patient's blood and using a modified virus to insert working copies of the beta-globin gene into the cells. When these altered cells are given back to the patient, they are transported in the bloodstream to the bone marrow where they start to make healthy red blood cells that produce beta-globin. The effects of this treatment are expected to last for the patient's lifetime.

Zynteglo was granted conditional marketing authorisation. This means that there is more evidence to come about the medicine, which the company is required to provide. EMA regularly reviews any new information in order to update the product information and conditions of use.