12 March 2021
EMA/127031/2021

Review of thalassaemia medicine Zynteglo started

EMA has begun a safety review of the medicine Zynteglo, a gene therapy authorised to treat the rare blood condition beta thalassaemia.

The review follows a case of acute myeloid leukaemia, a cancer of the blood, in a patient treated with a related investigational medicine, bb1111. This medicine uses the same modified virus (known as a viral vector) as Zynteglo, to deliver a gene into body cells. However, bb1111 is under development to treat sickle cell disease rather than thalassaemia. As well as the leukaemia case, 2 other patients given bb1111 developed another blood disorder, myelodysplastic syndrome, which progressed to leukaemia in one of them.

So far no cases of leukaemia have been reported with Zynteglo itself. However, the possibility that this type of treatment could cause a blood cancer (insertional oncogenesis) had been recognised as a potential risk when it was authorised, and patients who receive the medicine are followed up long-term and monitored in a registry. Given this, and since bb1111 works in the same way, the company responsible for developing both medicines has paused supply of Zynteglo while the evidence is examined to understand whether the cancer might be related to their treatment. No other authorised medicines use the same viral vector, which is based on a type of virus called a lentivirus.

EMA's safety committee, PRAC, will now examine the evidence thoroughly at EU level, working closely with experts from EMA's Committee for Advanced Therapies (CAT), which is responsible for assessing this type of medicine, and decide on any relevant regulatory action for Zynteglo.

Zynteglo is currently only available from 2 centres in the EU, and only one patient has received the medicine outside the context of clinical studies. If patients treated with Zynteglo or bb1111 have any concerns they should contact the doctor supervising their treatment.

More about the medicine

Due to an altered gene, 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 modified cells are given back to the patient, they are transported in the bloodstream to the bone marrow where they start to make red blood cells that are able to produce beta-globin. The effects of this treatment are expected to last for the patient's lifetime.

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Zynteglo was granted conditional marketing authorisation in May 2019. 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.

More about the procedure

The review of Zynteglo was initiated on 18 February, 2021, at the request of the European Commission, under Article 20 of Regulation (EC) No 726/2004.

The review is being carried out by the Pharmacovigilance Risk Assessment Committee (PRAC), the Committee responsible for the evaluation of safety issues for human medicines, which will make a set of recommendations, to be shared with the Committee for Advanced Therapies. The recommendations will then be sent to the Committee for Medicinal Products for Human Use (CHMP), responsible for questions concerning medicines for human use, which will adopt the Agency's opinion. The CHMP opinion will then be forwarded to the European Commission, which will issue a final legally binding decision applicable in all EU Member States in due course.