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Media and Public Relations

Press release

New gene therapy to treat rare inherited blood condition

EMA has recommended granting a marketing authorisation in the European Union for a genetically modified product for beta-thalassaemia, a rare inherited blood condition that causes severe anaemia. Zynteglo is intended for adult and adolescent patients 12 years and older who need regular blood transfusions to manage their disease and have no matching donor for a stem cell transplant.

Patients suffering from beta-thalassaemia cannot produce enough beta-globin, a key component of haemoglobin, the protein that carries the oxygen in the blood from the lungs to the rest of the body. As a consequence, they have far fewer red blood cells than normal and suffer from chronic severe anaemia.

These patients often require lifelong blood transfusions for their anaemia. Chronic transfusions can cause iron overload, hence patients need to take medication to remove excess iron. So far, the only curative treatment option is stem cell transplantation from a healthy donor. However, in the absence of a matched family donor, finding appropriate donors is often difficult and stem cell transplantation with unrelated donors is associated with severe side-effects and reactions. Patients who depend on blood transfusions to manage beta-thalassaemia therefore have an unmet medical need for new treatments.

Zynteglo is a new therapeutic option for patients for whom a related donor for stem cell transplantation is not available. Using a lentiviral viral vector, it adds functional copies of a modified β-globin gene into a patient's own stem cells, thereby addressing the underlying genetic cause of the disease.

Zynteglo should only be administered in a qualified treatment centre by a physician with experience in stem cell transplantation and in the treatment of patients with beta-thalassaemia.

In the two main studies to demonstrate the effects of Zynteglo it was shown that the majority of patients who do not have a β0/β0 genotype treated with Zynteglo no longer needed regular blood transfusions.

The most common side effects were thrombocytopenia (low blood platelet count), abdominal pain, non-cardiac chest pain, pain in the extremities, dyspnoea and hot flush.

Since Zynteglo addresses an unmet medical need, it benefited from PRIME, EMA's platform for early and enhanced dialogue with developers of promising new medicines. This interaction led to a more robust application package to demonstrate the medicine's benefits and risks, which allowed accelerated

assessment of Zynteglo in 150 days, the fastest advanced-therapy medicinal product (ATMP) review time to date.

Zynteglo was designated as an orphan medicinal product on 24 January 2013. During the development protocol assistance was received on multiple occasions to obtain guidance on the quality aspects and the clinical study programme, including the demonstration of significant benefit.

Because Zynteglo is an ATMP, it was assessed by the Committee for Advanced Therapies (CAT), the Agency's expert committee for cell- and gene-based medicines.

On the basis of the CAT's assessment and positive opinion, EMA's committee for human medicines (CHMP) recommended a conditional approval for this medicine. This is one of the EU's regulatory mechanisms to facilitate early access to medicines that fulfil an unmet medical need. This type of approval allows the Agency to recommend a medicine for marketing authorisation with less complete data than normally expected, in cases where the benefit of a medicine's immediate availability to patients outweighs the risk inherent in the fact that not all the data are yet available.

The opinion adopted by the CHMP is an intermediary step on Zynteglo's path to patient access. The opinion will now be sent to the European Commission for the adoption of a decision on an EU-wide marketing authorisation. Once the marketing authorisation has been granted, decisions about price and reimbursement will take place at the level of each Member State, taking into account the potential role/use of this medicine in the context of the national health system of that country.

Notes

1. This press release, together with all related documents, is available on the Agency's website.
2. The applicant for Zynteglo is bluebird bio (Netherlands) B.V.
3. As always at time of approval, EMA's committee for orphan medicines (COMP) will review the orphan designation to determine whether the information available to date allows maintaining Zynteglo's orphan status and granting this medicine ten years of market exclusivity.
4. More information on the work of the European Medicines Agency can be found on its website: www.ema.europa.eu

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