



28 March 2019  
EMA/CHMP/166977/2019  
Committee for Medicinal Products for Human Use (CHMP)

## Summary of opinion<sup>1</sup> (initial authorisation)

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### Zynteglo

#### Autologous CD34+ cells encoding $\beta^{A-T87Q}$ -globin gene

On 28 March 2019, the Committee for Medicinal Products for Human Use (CHMP) on the basis of the draft Committee for Advanced Therapies opinion, adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Zynteglo, intended for the treatment of transfusion-dependent  $\beta$ -thalassaemia (TDT).

As Zynteglo is an advanced therapy medicinal product, the CHMP's positive opinion is based on an assessment by the Committee for Advanced Therapies. Zynteglo was designated as an orphan medicinal product on 24 January 2013 and was reviewed under EMA's accelerated assessment programme. The applicant for this medicinal product is bluebird bio (Netherlands) B.V.

Zynteglo will be available as a  $1.2-20 \times 10^6$  cells/ml dispersion for infusion. The active substance of Zynteglo is an autologous CD34+ cell enriched population that contains hematopoietic stem cells (HSC) transduced with lentiviral vector (LVV) encoding the  $\beta^{A-T87Q}$ -globin gene. Zynteglo is a gene therapy medicinal product that adds functional copies of a modified  $\beta$ -globin gene into the patients' haematopoietic stem cells through transduction of autologous CD34+ cells with BB305 lentivirus vector, thereby addressing the underlying genetic cause of the disease.

The benefits with Zynteglo are its ability to enable  $\beta^{A-T87Q}$ -globin expression which is designed to correct the  $\beta/\alpha$ -globin imbalance in erythroid cells of patients with TDT and has the potential to increase total haemoglobin to normal levels and eliminate dependence on chronic red blood cell transfusions. The most common side effects are thrombocytopenia, abdominal pain, non-cardiac chest pain, pain in the extremities, dyspnoea and hot flush.

The full indication is: "treatment of patients 12 years and older with transfusion-dependent  $\beta$ -thalassaemia (TDT) who do not have a  $\beta^0/\beta^0$  genotype, for whom haematopoietic stem cell (HSC) transplantation is appropriate but a human leukocyte antigen (HLA)-matched related HSC donor is not available (see sections 4.4 and 5.1)." It is proposed that Zynteglo be administered in a qualified treatment centre by a physician with experience in HSC transplantation and in the treatment of patients with TDT.

Detailed recommendations for the use of this product will be described in the summary of product

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<sup>1</sup> Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion



characteristics (SmPC), which will be published in the European public assessment report (EPAR) and made available in all official European Union languages after the marketing authorisation has been granted by the European Commission.

Medicinal product no longer authorised