



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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Patient Health Protection

## Scientific recommendation on classification of advanced therapy medicinal products

Article 17 – Regulation (EC) No 1394/2007

**Disclaimer:** This document is a summary for public release of a scientific recommendation on classification of advanced therapy medicinal products. The original text adopted by the Committee for Advanced Therapies (CAT) has been redacted to delete commercially confidential information.

The present scientific recommendation refers exclusively to the case as presented to the European Medicines Agency (EMA) without prejudice to future evaluations by the Agency.

This scientific recommendation is not binding and is without prejudice to any decision taken by Member State competent authorities on matters falling within their own remits.

### **Short descriptor (or name when available) of the proposed active substance**

Autologous CD34+ haematopoietic stem cells (HSCs) transduced with lentiviral vector LentiGlobin encoding the human  $\beta$ A-T87Q-globin gene.

### **Brief description of the proposed finished product**

LentiGlobin transduced autologous CD34+ HSCs

### **Proposed indication**

beta-thalassemia major and intermedia, sickle cell anemia.

### **EMA/CAT comment**

#### ***Consideration of Article 1(2) of Directive 2001/83/EC (definition of medicinal product – see Annex A)***

- The product can be considered a 'substance' in the meaning of the pharmaceutical legislation (in accordance with article 1(3) of Directive 2001/83/EC), administered to humans with a view of modifying physiological functions by exerting a metabolic action



- The product is presented as having properties for treating disease in human being.
- According to Article 1(2), the restoration, correction or modification of the physiological function is to be mediated by the substances that exert "a pharmacological, immunological or metabolic action". As the product consists of autologous CD34+ HCS transduced with lentiviral vector Lentiglobin encoding the human beta-A-T87Q- globin gene, it can be agreed that the product acts via metabolic means.

***Fulfilment of Article 2(1) of Regulation (EC) No 1394/2007 (definition of advanced therapy medicinal product – see Annex A)***

- The product is a biological medicinal product in that it contains human (autologous) hematopoietic CD34+ cells transduced with a lentiviral vector
- The product consists of an active substance which contains recombinant nucleic acid (lentiviral vector encoding the human  $\beta^{A-T87Q}$ -globin gene)
- The product is intended for administration to human beings with a view to adding a genetic sequence (the human  $\beta^{A-T87Q}$ -globin gene)
- The therapeutic effect of the product relates directly to the product of genetic expression of this sequence, resulting in restored function of the concerned protein.

The medicinal product does not contain any medical device.

Based on the above considerations, it is considered that the product falls within the definition of an advanced therapy medicinal product and in particular within the definition of gene therapy medicinal product as provided in Article 2(1)(a) of Regulation (EC) No. 1394/2007.

**EMA/CAT conclusion**

On the basis that:

- The product is a biological medicinal product in that it contains human (autologous) hematopoietic CD34+ cells transduced with a lentiviral vector
- The product consists of an active substance which contains recombinant nucleic acid (lentiviral vector encoding the human  $\beta^{A-T87Q}$ -globin gene)
- The product is intended for administration to human beings with a view to adding a genetic sequence (the human  $\beta^{A-T87Q}$ -globin gene)
- The therapeutic effect relates directly to the product of genetic expression of this sequence, resulting in restored function of the concerned protein.

The EMA/CAT considers that the product falls within the definition of a gene therapy medicinal product as provided in Article 2(1)(a) of Regulation (EC) No 1394/2007