



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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Inspections, Human Medicines Pharmacovigilance & Committees Division

## 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.

It is stressed that the scientific recommendation on advanced therapy classification does not amount to any endorsement of the plausibility of the product, including the mode of action or therapeutic indication(s) claimed by the applicant.

### **Brief description (or name when available) of the active substance(s)**

Haematopoietic stem cells genetically modified to express a zinc finger nuclease which disrupts the enhancer of BCL11A expression.

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

Suspension of cells in a cryopreservation medium.

### **Proposed indication**

Treatment of  $\beta$ -thalassaemia.

### **EMA/CAT conclusion**

The procedure was finalised on 21 April 2016 for the following recommendation.

On the basis that:

- the product contains an active substance which contains a recombinant nucleic acid and is administered to humans with a view to deleting and/or modifying a genetic sequence;



- the therapeutic effect of the product relates directly to the product of genetic expression of the recombinant nucleic acid sequence it contains,

the EMA/CAT considers that the product falls within the definition of a gene therapy medicinal product, as provided in Article 2(1) of Regulation (EC) 1394/2007.