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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 Lenti-D encoding the human ABCD1 cDNA.

Brief description of the proposed finished product

Lenti-D transduced autologous CD34+ HSCs.

Proposed indication

Childhood cerebral adrenoleukodystrophy (CCALD).

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 administered to humans with a view of modifying physiological functions by exerting a metabolic action.

7 Westferry Circus • Canary Wharf • London E14 4HB • United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7051
E-mail info@ema.europa.eu **Website** www.ema.europa.eu

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+ HSCs transduced with lentiviral vector Lenti-D encoding the human ABCD1 cDNA, it can be agreed that the product acts via metabolic action.

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 Lenti-D encoding the normal human ABCD1 cDNA)
- The product is intended for administration to human beings with a view to adding a genetic sequence (the funtional human ABCD1 cDNA)
- The product relates directly to the product of genetic expression of this sequence, resulting in restored function of ALD protein (ALDP).

The medicinal product Lenti-D transduced autologous CD34+ HSCs 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, 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 Lenti-D encoding the normal human ABCD1 cDNA)
- The product is intended for administration to human beings with a view to adding a genetic sequence (the normal human ABCD1 cDNA)
- The therapeutic effect of the product relates directly to the product of genetic expression of this sequence

Based on the above considerations, it is considered that the product falls within the definition of an advanced therapy medicinal product, in particular of a gene therapy medicinal product.

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.