31 January 2018
EMA/799372/2017 Corr.[1]
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)

Cultured human retinal pigment epithelial cells genetically modified to express human factor IX protein.

Brief description of the finished product

Encapsulated human retinal pigment epithelial cells genetically modified to express human factor IX protein.

Proposed indication

Treatment of Hemophilia B.

EMA / CAT conclusion

The procedure was finalised on 9 November 2017 for the following recommendation.

On the basis that:

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- the product fulfils the definition of a biological medicinal product;
- the product contains a recombinant nucleic acid administered to human beings with a view to adding a genetic sequence;
- the therapeutic effect relates directly to the product of genetic expression of this sequence;
- the product is used on patients with a view to treat Hemophilia B;
- the product incorporates, as an integral part of the product, one or more medical devices within the meaning of Article 1(2)(a) of Directive 93/42/EEC,

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

1 Outcome of the scientific classification corrected, 'combined advanced therapy medicinal product' added