13 November 2017
EMA/751492/2017
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)

Adeno-associated viral vector serotype 8 containing the human low-density lipoprotein receptor (LDLR) gene.

Brief description of the finished product

Suspension of product vector particles supplied in a vial.

Proposed indication

Treatment of homozygous familial hypercholesterolemia caused by mutations in the LDLR gene.

EMA / CAT conclusion

The procedure was finalised on 17 October 2017 for the following recommendation.

On the basis that:

- the product contains a biological medicinal product as the active substance;

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- the active substance is a recombinant nucleic acid administered to human beings with a view to adding a genetic sequence;
- its therapeutic effect relates directly to the product of the genetic expression of this sequence,

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) of Regulation (EC) 1394/2007.