



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Procedure Management & Committees Support Division
Scientific Committee Support Department

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

Brief description (or name where available) of the active substance(s)

Adeno-associated virus serotype 2 based vector containing the human RPE65 gene expression cassette

Brief description of the finished product

Solution of recombinant AAV2 vectors supplied in a vial

Proposed indication

Treatment of inherited retinal degeneration due to autosomal recessive RPE65 gene mutations

EMA/CAT conclusion

The committee adopted on 29th January 2016 the following scientific recommendation.

On the basis that:

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

