



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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Procedure Management & Business 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**

The product is composed of two recombinant Adeno-Associated Viral Vectors derived from wild-type AAV2/5. The expression cassettes contain DNA encoding an RNA interference (RNAi) suppressor molecule, designed to suppress both mutant and wild-type rhodopsin gene transcripts.

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

The product will be supplied as a suspension containing both AAV-R and AAV-S vectors. The pharmaceutical form is a solution for injection supplied in a vial.

### **Proposed indication**

Treatment of autosomal dominant rhodopsin-linked retinitis pigmentosa.

### **EMA/CAT conclusion**

On the basis that:

- (a) The product contains a biological medicinal product as the active substance
- (b) The product contains an active substance which contains a recombinant nucleic acid administered to human beings with a view to regulating, repairing, adding a genetic sequence



(c) its therapeutic, prophylactic or diagnostic effect relates directly to the recombinant nucleic acid sequence it contains, or to the product of genetic expression of this sequence.  
EMA/CAT considers that the product falls within the definition of Gene Therapy Product as provided in Article 2(1)(a) of Regulation (EC) No 1394/2007.