19 July 2017
EMA/417253/2017
Inspections Human Medicines Pharmacovigilance Division
Committees and Inspections 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.

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)

Recombinant adeno-associated virus (AAV) pseudotyped with viral capsid from serotype 5 which holds a construct that contains two guide ribonucleic acids (gRNAs) sequences (CEP290-64 and CEP290-323) driven by human U6 promoter elements and the clustered regularly interspaced short palindromic repeats (CRISPR)-associated protein 9 (Cas9) gene.

Brief description of the finished product

Adenovirus-associated viral vector serotype 5 containing CRISPR Cas9 and guide RNAs targeting intron 26 of the CEP290 gene.

Proposed indication

For the treatment of patients aged 3 years and older with Leber Congenital Amaurosis type 10 (LCA10) caused by a homozygous or compound heterozygous intron 26 mutation, c.2991+1655 A>G, in the CEP290 gene.

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EMA/CAT conclusion

The procedure was finalised on 6 June 2017 for the following recommendation.

On the basis that the product:

- contains an active substance that contains a recombinant nucleic acid of biologic origin;
- is intended to be administered to human beings with a view to repairing, replacing, or adding a genetic sequence;
- its recombinant therapeutic effect relates directly to the recombinant nucleic acid sequence it contains, or to the product of genetic expression of this sequence,

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