6 June 2018
EMA/381214/2018
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)

Exosomes carrying recombinant mRNA encoding for the cystic fibrosis transmembrane conductance regulator protein and microRNA-17.

Brief description of the finished product

Suspension for aerosol delivery via a nebuliser device.

Proposed indication

Treatment of cystic fibrosis.

EMA/CAT conclusion

The procedure was finalised on 26 April 2018 for the following recommendation.

On the basis that the product:

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- is a biological medicinal product that contains active substances that consist of recombinant nucleic acids;
- the product is intended to be administered to human beings with a view to adding and regulating genetic sequences;
- its therapeutic effect relates directly to the recombinant nucleic acid sequence it contains, and to the product of genetic expression of the second 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.