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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)

DNA delivery vector with similar structure-function than bacteriophage capsids (i.e.. protein-based vectors able to recognize a bacterial host and inject it with the genetic material they carry) carrying a recombinant DNA payload that encodes a RNA-guided nuclease, CRISPR Cas9 or equivalent, targeting shiga-toxin genes.

Brief description of the finished product

Suspension of a multivalent mix of purified active substances formulated in a hydrogel form to allow oral administration in paediatric populations. The formulation will consist in a modified release dosage form that ensures gastroresistant properties, and effective delivery of the active substances at the intended site of action (distal small and large intestine).

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Proposed indication

Treatment of infections mediated by Shiga-Toxin producing Escherichia coli that have already colonized the patient's intestine at the time of diagnosis and translate clinically into bloody diarrhoea (> 90% cases). The product is intended to resolve bloody diarrhoea and prevent the occurrence of a Haemolytic Uremic Syndrome, a severe complication leading to kidney failure and death or life-long sequelae.

EMA/CAT conclusion

The procedure was finalised on 20 December 2018 for the following recommendation.

On the basis that:

- the product contains an active substance which consists of 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 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.