



09 September 2018  
EMA/608944/2018  
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)

The active substance is a patient-specific mRNA molecule encoding neo-epitopes that are specifically derived from tumour tissue. The mRNA molecules are produced by *in vitro* transcription.

### Brief description of the finished product

The product is presented as an aqueous solution of a nanoparticulate mRNA lipoplex formulation.

### Proposed indication

Treatment of locally advanced or metastatic tumours.

### EMA/CAT conclusion

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

On the basis that:



- the product contains an active substance that consists of a recombinant nucleic acid molecule of biologic origin;
- the product is intended to be 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.