



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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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)

Nuclease resistant, synthetic double-stranded small interfering RNA (siRNA).

Brief description of the finished product

Suspension for solution for infusion and powder for concentrate for solution for infusion.

Proposed indication

Treatment of hepatic fibrosis.

EMA/CAT conclusion

The procedure was finalised on 14 September 2017 for the following recommendation.

On the basis that the product:



- does not contain an active substance which contains or consists of a recombinant nucleic acid administered to human beings with a view to regulating/repairing/adding/deleting a genetic sequence;
- does not contain / consist of cells or tissues that have been subject to substantial manipulation so that biological characteristics, physiological functions or structural properties relevant for the intended clinical use have been altered;
- does not contain/consist of cells or tissues that are intended to be used for a different essential function in the recipient and the donor,

the EMA/CAT considers that the product does not fall within the definition of an advanced therapy medicinal product as provided in Article 2(1) of Regulation (EC) 1394/2007.