



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Patient Health Protection

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.

This scientific recommendation is not binding and is without prejudice to any decision taken by Member State competent authorities on matters falling within their own remits.

Short descriptor (or name when available) of the proposed active substance

The active substance is autologous Mesenchymal Stromal Cells secreting NeuroTrophic Factors (MSC-NTF).

The product consists of bone-marrow-derived MSC which have been propagated *ex vivo*, and induced to secrete NTF, such as Glial Derived Growth Factor (GDNF) and Brain Derived Growth Factor (BDNF).

Brief description of the proposed finished product

The final product is injected as a cell suspension intrathecally or intramuscularly.

Proposed indication

Amyotrophic lateral sclerosis (ALS)



EMA/CAT comment

Consideration of Article 1(2) of Directive 2001/83/EC (definition of medicinal product – see Annex A)

- The product consists of human autologous mesenchymal stromal cells which can be considered as 'substance' administered with a view for treating or preventing disease in human beings.
- The product is presented to have properties for correcting or modifying physiological functions by exerting a pharmacological, immunological or metabolic action.

Fulfilment of Article 2(1) of Regulation (EC) No 1394/2007 (definition of advanced therapy medicinal product – see Annex A)

- The product manufacturing process includes *ex vivo* tissue culture - expansion of cells and induction of neuroprotective factor secretion - which is considered a substantial manipulations,
- The product is intended to treat and prevent a disease.

Based on the above considerations, it is considered that the product falls within the definition of an advanced therapy medicinal product, as provided in Article 2(1)(a) of Regulation (EC) No 1394/2007.

EMA/CAT conclusion

On the basis that:

- the product consists of cells 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,
- the product is presented as having properties for, or is used in or administered to human beings with a view to treating and preventing a disease through the pharmacological, immunological or metabolic action,

the EMA/CAT considers that the product falls within the definition of a **somatic cell therapy medicinal product** as provided in Article 2(1)(a) of Regulation (EC) No 1394/2007 and defined in Part IV of Annex I to Directive 2001/83/EC.