EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

21 March 2016
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Procedure Management & Committees Support Division
Scientific Committee Support Department

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

Brief description (or name where available) of the active substance(s)

Adeno-associated virus serotype 8 vector encoding human ornithine transcarbamylase

Brief description of the finished product

Concentrate for solution for infusion

Proposed indication

Treatment of ornithine transcarbamylase deficiency

EMA / CAT conclusion

The committee adopted on 21st December 2015 the following scientific recommendation.

On the basis that:

- The product contains a biological medicinal product as the active substance;
- The active substance is 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 the genetic expression of this sequence.

The EMA/CAT considers that the product falls within the definition of a gene therapy medicinal product.

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