EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

29 October 2014
EMA/556494/2015
Procedure Management & Business 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)

AAV vector carrying an expression cassette for photoactivable enhanced halorhodopsin protein from Natronomonas pharaonis (eNpHR).

Brief description of the finished product

Adeno-associated virus (AAV) vector carrying a gene for bacterial halorhodopsin

Proposed indication

Intended for the treatment of Retinitis Pigmentosa.

EMA/CAT conclusion

On the basis that:

-the product consists of an Adeno-Associated Viral Vector containing DNA encoding a bacterial halorhodopsin protein;

-the product is intended to be used to compensate, or bypass, a genetic defect responsible for retinitis pigmentosa by providing an alternative pathway for light-induced stimulation of photoreceptor cells;

-The mechanism of action is given by expression of the encoded halorhodopsin for treatment of retinitis pigmentosa;

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The EMA/CAT considers that the product falls within the definition of a gene therapy medicinal product as provided for in Article 2(1)(a) of Regulation (EC) No. 1394/2007.