Title (en)
ADENO-ASSOCIATED VIRAL VECTORS FOR THE EXPRESSION OF DYSFERLIN

Title (de)
ADENO-ASSOZIIERTE VIRUSVEKTOREN ZUR EXPRESSION VON DYSFERLIN

Title (fr)
VECTEURS VIRAUX ADÉNO-ASSOCIÉS POUR L'EXPRESSION DE LA DYSFERLINE

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Abstract (en)
[origin: WO2009016326A2] The present invention relates to a composition comprising: a first adeno-associated viral (AAV) vector comprising: i) a 5'ITR (Inverted Terminal Repeat) sequence of AAV; ii) a portion of gene placed under the control of a promoter; iii) a sequence comprising a splice donor site; iv) a 3'ITR sequence of AAV; and/or a second adeno-associated viral (AAV) vector comprising: v) a 5'ITR (Inverted Terminal Repeat) sequence of AAV; vi) a sequence comprising a splice acceptor site; vii) a portion of gene; viii) a 3'ITR sequence of AAV. The combination of the portions of gene carried by the first and second AAV vectors comprises an open reading frame which encodes a functional dysferlin. In addition, the combination of the sequence comprising the splice donor site and the sequence comprising the splice acceptor site contains all the elements necessary for the splicing, advantageously derived from a natural intron of the dysferlin gene.

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