Title (en)

SELF-DELETING VECTORS FOR GENE THERAPY

Title (de)

SICH SELBST ENTFERNENDE VEKTOREN FUER DIE GENTHERAPIE

Title (fr)

VECTEURS A AUTO-SUPPRESSION POUR THERAPIE GENIQUE

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Application

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Abstract (en)

[origin: DE19530412A1] The invention involves the development of vectors for somatic gene therapy. The vectors transduce a complete transcriptional unit containing a promoter, a protein coding sequence and a polyadenylation sequence into the genome of mammalian cells. Upon integration, the vectors delete most viral and non-viral sequences unrelated to transcriptional unit thus avoiding common problems encountered with conventional retrovirus vectors such as repression of gene expression by transcriptional silencing, mobilization of endogenous retroviruses, activation of oncogenes or development of an immune response. The invention exploits (i) the natural life cycle of retroviruses, involving duplication of the terminal control regions U5 and U3 to generate long terminal repeats (LTR) and (ii) the ability of site specific recombinases to excise any sequences positioned between two specific target sequences from the mammalian genome. Thus, the retroviruses of the invention transduce the coding sequences of a site specific recombinase and at least one recombinase-specific target sequence into the genome along with the transcriptional unit expressing a therapy gene.

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