

Title (en)  
MINI-ADENOVIRAL VECTOR

Title (de)  
MINI-ADENOVIRALER VEKTOR

Title (fr)  
VECTEUR MINI-ADENOVIRAL

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Application  
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Abstract (en)  
[origin: WO9745550A2] This invention is related to adenoviral (Ad) vectors and their applications in the field of genetic medicine, including gene transfer, gene therapy, and gene vaccination. More specifically, this invention is related to the Ad vectors that carry the minimal cis-element of the Ad genome (mini-Ad vector) and are capable of delivering transgenes and/or heterologous DNA up to 36 kb. The generation and propagation of the mini-Ad vectors require trans-complementation of a packaging-attenuated and replication-defective helper Ad (helper) in an Ad helper cell line. This invention further comprises a methodology for generating a mini-adenoviral (mini-Ad) vector for use in gene therapy of hemophilia and animal test systems for in vivo evaluation of the Ad vectors. More specifically, this invention describes factor VIII (FVIII) Ad vectors that only contain minimal cis-element of the Ad genome (so-called mini-Ad) and comprise a human FVIII cDNA with other supporting DNA elements up to 36 kb. The FVIII mini-Ad can be generated and preferentially amplified through the assistance of a packaging-attenuated helper Ad and a helper cell line. This invention also reports designs and methods for producing transgenic mouse models that can be used for in vivo testing the mini-Ad.

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