

Title (en)  
REGULATED EXPRESSION OF TRANSGENES IN THE CENTRAL NERVOUS SYSTEM OF MAMMALS

Title (de)  
REGULIERTE EXPRESSION VON TRANSGENEN IM ZENTRALNERVENSYSTEM VON SÄUGERN

Title (fr)  
EXPRESSION REGULEE DE TRANSGENES DANS LE SYSTEME NERVEUX CENTRAL DE MAMMIFERES

Publication  
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Application  
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Abstract (en)  
[origin: WO2006063247A2] Recombinant adeno-associated virus (rAAV) vectors are provided for delivery of regulatable transgenes to the central nervous system (CNS) of a mammal. Also provided are methods of treatment of subjects with neurodegenerative disorders using the vectors, and kits for constructing or using the vectors or performing the methods of the invention. Transgene sequences are expressed from a promoter/enhancer region comprising one or more binding sites for a transcription factor that is responsive to a small molecule inducer. Both the transgene construct and a construct comprising the transcription factor are delivered to the target cells. The regulatable transgene may be delivered on the same rAAV vector as the transcription factor, or on a separate vector. The transcription factor may comprise two polypeptide chains, e.g. a DNA binding domain and a transcription activation domain, that form an active dimer in the presence of a dimerizer such as rapamycin or a non-immunogenic analog thereof. Vectors, methods and kits of the invention may be used to deliver genes such as AADC or GDNF to the brain of a subject with a neurodegenerative disorder, such as Parkinson's disease, where the expression of AADC or GDNF in the brain can subsequently be regulated by treatment of the subject with rapamycin or a rapamycin analog.

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