RECOMBINANT ADENO-ASSOCIATED VIRUS FOR TREATMENT OF GRN-ASSOCIATED ADULT-ONSET NEURODEGENERATION

ABSTRACT

A recombinant AAV (rAAV) suitable for use in treating adult onset neurodegeneration caused by granulin (GRN) haploinsufficiency, such as progranulin (PGRN) - related frontotemporal dementia (FTD), is provided. The rAAV comprises (a) an adeno-associated virus 1 capsid, and (b) a vector genome packaged in the AAV capsid, said vector genome comprising AAV inverted terminal repeats, a coding sequence for human progranulin, and regulatory sequences which direct expression of the progranulin. Also provided are a method for treating a human patient with PGRN -FTD and other adult onset neurodegeneration caused by granulin (GRN) haploinsufficiencies, comprising delivering to the central nervous system (CNS) a recombinant adeno-associated virus (rAAV) having an adeno-associated virus 1 (AAV1) capsid, said rAAV further comprising a vector genome packaged in the AAV capsid, said vector genome comprising AAV inverted terminal repeats, a coding sequence for human progranulin, and regulatory sequences which direct expression of the progranulin.

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