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

ABSTRACT

A therapeutic regimen useful for treatment of adult-onset neurodegenerative disease in a human patient comprising administration of a recombinant adeno-associated virus (AAV) vector having an AAV1 capsid and a vector genome comprising a progranulin (GRN) coding sequence is provided. Also provided are compositions comprising a recombinant AAV vector and methods of treating adult-onset neurodegenerative disease in a patient comprising administration of the recombinant AAV vector.

Figure for publication: None