COMPOSITIONS USEFUL IN TREATMENT OF CDKL5 DEFICIENCY DISORDER (CDD)

ABSTRACT

Provided is a recombinant adeno-associated virus (rAAV) having an AAV capsid and a vector genome which comprises a nucleic acid sequence encoding a functional CDKL5 (hCDKLK5). Also provided are a production system useful for producing the rAAV, a pharmaceutical composition comprising the rAAV, and a method of treating a subject having CDD, or ameliorating symptoms of CDD, or delaying progression of CDD via administrating an effective amount of the rAAV to a subject in need thereof.

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