COMPOSITIONS USEFUL IN TREATMENT OF RETT SYNDROME

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 human methyl-CpG-binding protein 2 (hMECP2). Also provided is a production system useful for producing the rAAV, a pharmaceutical composition comprising the rAAV, and a method of treating a subject having Rett Syndrome, or ameliorating symptoms of Rett Syndrome, or delaying progression of Rett Syndrome via administrating an effective amount of the rAAV to a subject in need thereof.

FIG. 1