AAV VECTORS FOR RETINAL AND CNS GENE THERAPY

ABSTRACT

Provided herein are improved rAAV (*e.g.*, rAAV2, rAAVrh8R, *etc*.) for enhanced gene therapy of ocular disorders or CNS disorders wherein the rAAV comprise one or more substitutions of amino acids that interact with heparan sulfate proteoglycan. The invention provides methods for improved transduction of retinal cells and methods for treating ocular diseases with improved compositions of rAAV particles. Further provided herein are improved recombinant adeno-associated virus (rAAV) (*e.g.*, rAAV2, rAAVrh8R, *etc*.) for enhanced gene therapy of disorders of the CNS. The invention provides methods for delivering the rAAV to the CNS, methods for treating disorders of the CNS with improved compositions of rAAV particles, and kits for delivering the rAAV to the CNS and/or treating a CNS disorder.

[Figure 8]