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(54) Title: GENE THERAPY FOR NEUROMUSCULAR AND NEUROMOTOR DISORDERS

(57) Abstract: The invention provides methods of screening for capsid-encoding nucleotide sequences of adeno-associated virus ("AAV") particles capable of infecting a subject's neurons, or sub-compartments thereof, involving neurons derived from induced pluripotent stem cells or embryonic stem cells. The invention also provides AAV capsids, capsid-encoding nucleotide sequences, expression vectors, viral particles, cells and kits for use in methods of treating neuromuscular or neuromotor disorders, such as spasticity. Sequences generated from the methods may lead to new gene therapies targeted to select populations of neurons, such as motor neurons innervating muscle cells, thus providing high specificity that may be personalised to the subject.

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