CLAIMS

What is claimed is:

1. Use of a recombinant adeno-associated virus (rAAV) comprising:

(i) an isolated nucleic acid comprising a transgene encoding one or more gene products of interest; and

(ii) an adeno-associated acid (AAV) capsid protein having the sequence set forth in SEQ ID NO: 1,

in the manufacture of a medicament for the treatment of a disease or disorder associated with reduced expression, lack of expression or dysfunction of the one or more gene products of interest, wherein the medicament is to be intracranially administered to a subject to deliver the transgene to a target cell in the subject.

2. The use of claim 1, wherein the intracranial administration comprises intrahippocampal injection.

3. The use of claim 1 or 2, wherein the target cell is a central nervous system (CNS) cell.

4. The use of any one of claims 1 to 3, wherein the CNS cell is a neuron, oligodendrocyte, astrocyte, or microglial cell.

5. The use of any one of claims 1 to 4, wherein the subject is a mammal, optionally wherein the mammal is a human.

6. The use of any one of claims 1 to 5, wherein the subject is characterized by production of anti-AAV2 antibodies.

7. The use of claim 6, wherein after administration of the rAAV, the subject does not elicit a neutralizing immune response against the rAAV.

8. The use of any one of claims 1 to 7, wherein the isolated nucleic acid comprises AAV inverted terminal repeats (ITRs) flanking the transgene.

9. The use of any one of claims 1 to 8, wherein the nucleic acid sequence encoding the one or more gene products is operably linked to a promoter.

10. The use of any one of claims 1 to 9, wherein the one or more gene products comprise a protein or an inhibitory nucleic acid.

11. Use of a recombinant adeno-associated virus (rAAV) comprising:

(i) an isolated nucleic acid comprising a transgene encoding one or more gene products of interest; and

(ii) an adeno-associated acid (AAV) capsid protein having the sequence set forth in SEQ ID NO: 1,

in the manufacture of a medicament for the treatment of a disease or disorder associated with reduced expression, lack of expression or dysfunction of the one or more gene products of interest, wherein the medicament is to be intravenously administered to a subject to deliver the transgene to a target cell in the subject,

wherein the administration results in the rAAV crossing the blood brain barrier (BBB) of the subject.

12. The use of claim 11, wherein the target cell is a central nervous system (CNS) cell.

13. The use of claim 12, wherein the CNS cell is a neuron, oligodendrocyte, astrocyte, or microglial cell.

14. The use of any one of claims 11 to 13, wherein the administration results in decreased transduction of liver cells relative to administration of an rAAV having an AAV2 capsid protein

15. The use of any one of claims 11 to 14, wherein the subject is a mammal, optionally wherein the mammal is a human.

16. The use of any one of claims 11 to 15, wherein the subject is characterized by production of anti-AAV2 antibodies.

17. The use of claim 16, wherein after administration of the rAAV, the subject does not elicit a neutralizing immune response against the rAAV.

18. The use of any one of claims 11 to 17, wherein the isolated nucleic acid comprises AAV inverted terminal repeats (ITRs) flanking the transgene.

19. The use of any one of claims 11 to 18, wherein the nucleic acid sequence encoding the one or more gene products is operably linked to a promoter.

20. The use of any one of claims 11 to 19, wherein the one or more gene products comprise a protein or an inhibitory nucleic acid.