CLAIMS

What is claimed is:

1. An Adeno-Associated Virus (AAV) transfer cassette comprising, from 5' to 3':
a 5' inverted terminal repeat (ITR);
a chicken beta-actin promoter;
a transgene;
a polyadenylation signal; and
a 3' ITR;
wherein the transfer cassette comprises an intronic sequence; and
wherein the transgene encodes the NPC1 protein.

2. The AAV transfer cassette of claim 1, wherein the 5' ITR comprises the sequence of SEQ ID NO: 3.

3. The AAV transfer cassette of claim 1, wherein the 3' ITR comprises the sequence of SEQ ID NO: 4.

4. The AAV transfer cassette of claim 1, wherein the promoter comprises the sequence of SEQ ID NO: 5.

5. The AAV transfer cassette of claim 1, wherein the intronic sequence is an SV40 intron.

6. The AAV transfer cassette of claim 1, wherein the intronic sequence comprises the sequence of SEQ ID NO: 10.

7. The AAV transfer cassette of claim 1, wherein the NPC1 protein is the human NPC1 protein.

8. The AAV transfer cassette of claim 1, wherein the NPC1 protein comprises the sequence of SEQ ID NO: 1.

9. The AAV transfer cassette of claim 1, wherein the transgene comprises the sequence of SEQ ID NO: 2.

10. The AAV transfer cassette of claim 1, wherein the polyadenylation signal is the SV40 polyadenylation signal.

11. The AAV transfer cassette of claim 1, wherein the polyadenylation signal comprises the sequence of SEQ ID NO: 12.

12. The AAV transfer cassette of claim 1, wherein the cassette comprises an enhancer.

13. The AAV transfer cassette of claim 1, wherein the AAV transfer cassette comprises the sequence of SEQ ID NO: 14

14. The AAV transfer cassette of claim 1, wherein the AAV transfer cassette comprises the sequence of any one of SEQ ID NO: 15-19.

15. A plasmid comprising the AAV transfer cassette of any one of claims 1-14.

16. A nucleic acid comprising, from 5' to 3', a 5' inverted terminal repeat (ITR); a promoter; a transgene; a polyadenylation signal; and a 3' ITR; wherein the nucleic acid comprises an intronic sequence; wherein the transgene encodes the amino acid sequence of SEQ ID NO: 1.

17. A cell comprising the AAV transfer cassette of any one of claims 1-14, the plasmid of claim 15, or the nucleic acid of claim 16.

18. A recombinant AAV vector comprising a protein capsid and a nucleic acid encapsidated by the protein capsid:

wherein the nucleic acid comprises the AAV transfer cassette of any one of claims 1-14.

19. The recombinant AAV vector of claim 18, wherein the protein capsid comprises a capsid protein subunit that is from an AAV of any one of the following serotypes: AAV1, AAV2, AAV3, AAV4, AAV5, AAV6, AAV7, AAV8, AAV9, AAV10, AAV11, AAV12, AAVrh8, AAVrh10, AAVrh32.33, AAVrh74, Avian AAV or Bovine AAV.

20. The recombinant AAV vector of claim 18, wherein the protein capsid comprises a capsid protein subunit that has one or more amino acid mutations relative to a capsid protein subunit of any one of the following AAV serotypes: AAV1, AAV2, AAV3, AAV4, AAV5, AAV6, AAV7, AAV8, AAV9, AAV10, AAV11, AAV12, AAVrh8, AAVrh10, AAVrh32.33, AAVrh74, Avian AAV or Bovine AAV.

21. A method of producing a recombinant AAV vector, the method comprising contacting an AAV producer cell with the AAV transfer cassette of any one of claims 1-14, the plasmid of claim 15, or the nucleic acid of claim 16.

22. A recombinant AAV vector produced by the method of claim 21.

23. A composition comprising the AAV transfer cassette of any one of claims 1-14, the plasmid of claim 15, the nucleic acid of claim 16, the cell of claim 17, or the recombinant AAV vector of any one of claims 18-20 or 22.

24. A method for treating a subject in need thereof comprising administering to the subject an effective amount of the AAV transfer cassette of any one of claims 1-14, the plasmid of claim 15, the nucleic acid of claim 16, the cell of claim 17, or the recombinant AAV vector of any one of claims 18-20 or 22.

25. The method of claim 24, wherein the subject has NPC1.

26. The method of claim 24, wherein the subject is a human subject.

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