CLAIMS

What is claimed is:

1. An Adeno-Associated Virus (AAV) transfer cassette comprising, from 5' to 3':
a 5' inverted terminal repeat (ITR) comprising a sequence of SEQ ID NO: 3;
a promoter;
a transgene encoding a human Niemann-Pick Disease, type C1 (NPC1) protein comprising the sequence of SEQ ID NO: 1;
a polyadenylation signal; and
a 3' ITR comprising a sequence of SEQ ID NO: 4;
wherein the transfer cassette comprises a sequence of any one of SEQ ID NO: 14-17 and 19.

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

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

4. A plasmid comprising the AAV transfer cassette of claim 1.

5. A cell comprising the AAV transfer cassette of any one of claims 1-3, or the plasmid of claim 4.

6. 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 1-3.

7. The recombinant AAV vector of claim 6, 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.

8. The recombinant AAV vector of claim 6, 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.

9. 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 1-3, or the plasmid of claim 4.

10. A composition comprising the AAV transfer cassette of any one of claims 1-3, the plasmid of claim 4, the cell of claim 5, or the recombinant AAV vector of any one of claims 6-8.

11. Use of the AAV transfer cassette of any one of claims 1-3, the plasmid of claim 4, the cell of claim 5, or the recombinant AAV vector of any one of claims 6-8 in the manufacture of a medicament for treating Niemann-Pick Disease, type C1, in a subject in need thereof.

12. The use of claim 11, wherein the subject is a human subject.