

Claims

1. A nucleic acid molecule comprising a nucleic acid sequence at least 90% identical to the nucleic acid sequence of SEQ ID NO: 4.
2. The nucleic acid molecule of claim 1, comprising a nucleic acid sequence at least 95% identical to the nucleic acid sequence of SEQ ID NO: 4.
3. The nucleic acid molecule of claim 1 or 2 comprising a nucleic acid sequence of SEQ ID NO: 4.
4. A self-complementary recombinant adeno-associated virus 9 (scAAV9) comprising a nucleic acid molecule of any one of claims 1 to 3.
5. The scAAV9 of claim 4, wherein the scAAV9 comprising a single-stranded genome.
6. A rAAV particle comprising a nucleic acid molecule of any one of claims 1 to 3.
7. The rAAV particle of claim 6, wherein the rAAV particle comprising a single-stranded genome.
8. A composition comprising the scAAV9 of claim 4 or 5, the nucleic acid molecule of any one of claims 1 to 3, rAAV particle of claim 6 or 7 and a pharmaceutically acceptable excipient, carrier, or diluent.
9. The composition of claim 8, wherein the pharmaceutically acceptable excipient comprises a non-ionic low osmolar compound, a buffer, a polymer, a salt, or a combination thereof.
10. Use of a therapeutically effective amount of the scAAV9 of claim 4 or 5, the nucleic acid molecule of any one of claims 1 to 3, rAAV particle of claim 6 or 7, or the composition of claim 8 or 9 for the preparation of a medicament for the treatment of CLN3 Batten Disease.
11. The use of claim 10, wherein the medicament is formulated for administration via a route selected from the group consisting of intrathecal, intracerebroventricular, intraparenchymal, intravenous, and a combination thereof.
12. The use of claim 10, wherein the medicament is formulated for intrathecal administration.
13. The use of claim 10, wherein the medicament is formulated for intracerebroventricular administration.

14. The use of claim 10, wherein the medicament is formulated for intravenous administration.
15. The use of any one of claims 10-14, wherein the medicament comprises 1×10^{12} to 1×10^{15} viral genomes of the rAAV particle.
16. The use of any one of claims 10 to 15, wherein the medicament comprises 6×10^{13} to 1.2×10^{14} viral genomes of the rAAV viral particle.
17. The use of any one of claims 10 to 16, wherein administration of the medicament reduces one or more symptoms of CLN3-Batten Disease selected from:
 - (a) reduced or slowed lysosomal accumulation of autofluorescent storage material,
 - (b) reduced or slowed lysosomal accumulation of ATP Synthase Subunit C,
 - (c) reduced or slowed glial activation (astrocytes and/or microglia) activation,
 - (d) reduced or slowed astrocytosis,
 - (e) reduced or slowed brain volume loss measured by MRI,
 - (f) reduced or slowed onset of seizures,
 - (g) stabilization, reduced or delayed progression, or improvement in one or more of the UBDRS assessment scales, wherein the reduction, stabilization, or improvement is as compared to the subject prior to administration of the composition or to an untreated CLN3-Batten Disease patient.
18. The use of any one of claims 10 to 17, wherein the subject is placed in the Trendelenburg position after administration of the rAAV particle.
19. A composition suitable for treating of CLN3 Batten Disease, wherein the composition comprises a therapeutically effective amount of the scAAV9 of claim 4 or 5 1, the nucleic acid molecule of any one of claims 1 to 3, rAAV particle of claim 6 or 7 , or the composition of claim 8 or 9 for the preparation of a medicament for the treatment of CLN3 Batten Disease.
20. The composition of claim 19, wherein the composition is formulated for administration via a route selected from the group consisting of intrathecal, intracerebroventricular, intraparenchymal, intravenous, and a combination thereof.
21. The composition of claim 20, wherein the composition is formulated for intrathecal administration.
22. The composition of claim 20, wherein the composition is formulated for intracerebroventricular administration.

23. The composition of claim 20, wherein the composition is formulated for intravenous administration.
24. The composition of any one of claims 19 to 23, wherein the composition comprises 1×10^{12} to 1×10^{15} viral genomes of the rAAV particle.
25. The composition of any one of claims 19 to 23, wherein the medicament comprises 6×10^{13} to 1.2×10^{14} viral genomes of the rAAV particle.
26. The composition of any one of claims 19 to 25, wherein administration of the composition reduces one or more symptoms of CLN3-Batten Disease selected from:
- (a) reduced or slowed lysosomal accumulation of autofluorescent storage material,
 - (b) reduced or slowed lysosomal accumulation of ATP Synthase Subunit C,
 - (c) reduced or slowed glial activation (astrocytes and/or microglia) activation,
 - (d) reduced or slowed astrocytosis,
 - (e) reduced or slowed brain volume loss measured by MRI,
 - (f) reduced or slowed onset of seizures,
 - (g) stabilization, reduced or delayed progression, or improvement in one or more of the UBDRS assessment scales, wherein the reduction, stabilization, or improvement is as compared to the subject prior to administration of the composition or to an untreated CLN3-Batten Disease patient.