

Claims

1. Use of a recombinant adeno-associated virus (rAAV) AAVrh74.tMCK.hSCGA A in the manufacture of a medicament for treating muscular dystrophy in a subject in need thereof, wherein the rAAV is at a dose of about 5×10^{13} vg/kg to about 2×10^{14} vg/kg based on a supercoiled DNA or plasmid as the quantitation standard, and wherein the medicament is formulated for a systemic route of administration.
2. The use of claim 1, wherein the rAAV is at a dose about 5×10^{13} vg/kg, about 1×10^{14} vg/kg, or about 2×10^{14} vg/kg based on a supercoiled DNA or plasmid as the quantitation standard.
3. The use of any one of claims 1 or 2, wherein the rAAV comprises the scAAVrh74.tMCK.hSGCA construct nucleotide sequence with at least 90%, 91%, 92%, 93%, 94%, 95%, 96%, 97%, 98%, or 99% identity to SEQ ID NO: 4.
4. The use of claim 3, wherein the rAAV comprises the scAAVrh74.tMCK.hSGCA construct nucleotide sequence of SEQ ID NO: 4.
5. The use of any one of claims 1-4, wherein the level of alpha-sarcoglycan gene expression in a cell of the subject is increased after administration of the medicament as compared to the level of alpha-sarcoglycan gene expression before administration of the medicament; or wherein the serum CK level in the subject is decreased after administration of the medicament as compared to serum CK level before administration of the medicament; or wherein the number of alpha-sarcoglycan positive fibers in the muscle tissue of the subject is increased after administration of the medicament as compared to the number of alpha-sarcoglycan positive fibers before administration of the medicament.
6. The use of any one of claims 1-5, wherein the systemic route of administration is an intravenous route.
7. The use of any one of claims 1-6, wherein the medicament is formulated for administration by injection, infusion or implantation.
8. The use of any one of claims 1-7, wherein the medicament is formulated for administration by infusion.
9. The use of any one of claims 1-7, wherein the medicament is formulated for administration by an intravenous route through a peripheral limb vein.
10. The use of any one of claims 1-9, wherein the muscular dystrophy is limb-girdle muscular dystrophy.
11. The use of any one of claims 1-10, wherein the muscular dystrophy is limb-girdle muscular dystrophy type 2D (LGMD2D).

12. The use of any one of claims 1-11, wherein the subject is suffering from limb-girdle muscular dystrophy, and the medicament is formulated for intravenous infusion and the rAAV is at a dose of about 5×10^{13} vg/kg to about 2×10^{14} vg/kg based on a supercoiled DNA or plasmid as the quantitation standard, and wherein the rAAV comprises the scAAVrh74.tMCK.hSGCA construct nucleotide sequence of SEQ ID NO: 4.

13. Use of a scAAVrh74.tMCK.hSGCA construct in the manufacture of a medicament for the treatment of muscular dystrophy, wherein the medicament is for decreasing a serum CK level in a subject in need thereof, and wherein the scAAVrh74.tMCK.hSGCA construct comprises the nucleotide sequence of SEQ ID NO: 4.