Claims

1. Use of a recombinant adeno-associated virus (rAAV) AAVrh74.tMCK.hSCGA in the manufacture of a medicament for treating limb-girdle muscular dystrophy type 2D (LGMD2D) in a subject in need thereof, wherein the rAAV is at a dose between $5x10^{13}$ vg/kg and $2x10^{14}$ 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, and 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.

2. The use of claim 1, wherein the rAAV is at a dose selected from $5x10^{13}$ vg/kg, $1x10^{14}$ vg/kg, or $2x10^{14}$ vg/kg based on a supercoiled DNA or plasmid as the quantitation standard.

3. The use of claim 1, wherein the rAAV comprises the scAAVrh74.tMCK.hSGCA construct with a nucleotide sequence of SEQ ID NO: 4.

4. The use of any one of claims 1-3, 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.

5. The use of any one of claims 1-4, wherein the systemic route of administration is an intravenous route.

6. The use of any one of claims 1-5, wherein the medicament is formulated for administration by injection, infusion or implantation.

7. The use of any one of claims 1-6, wherein the medicament is formulated for administration by infusion.

8. The use of any one of claims 1-6, wherein the medicament is formulated for administration by an intravenous route through a peripheral limb vein.

9. The use of any one of claims 1-8, wherein the medicament is formulated for intravenous infusion and the rAAV is at a dose between $5x10^{13}$ vg/kg and $2x10^{14}$ 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.

10. Use of a scAAVrh74.tMCK.hSGCA construct in the manufacture of a medicament for the treatment of limb-girdle muscular dystrophy type 2D (LGMD2D), 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.