

analysis of citrate synthase suggests the presence of damaged mitochondria. These results suggest that mitochondrial abnormalities may be a secondary effect caused by the loss of the Anoctamin 5 protein. The experiments described herein demonstrate an attenuation of sarcolemmal patch repair in *Ano5*^{-/-} fibers that is rescued by viral expression of human ANO5 and find that the disruption of Ano5 perturbs the fusogenic quality of primary myoblasts.

[0010a] In one aspect, the present disclosure refers to the use of a recombinant AAV vector comprising 1) a nucleic acid sequence is at least 85% identical to the nucleic acid sequence of SEQ ID NO:1, 2) a nucleotide that hybridizes under stringent condition to the nucleotide sequence of SEQ ID NO: 1 or 3) a nucleic acid sequence encoding a protein that is at least 85% identical to the amino acid sequence of SEQ ID NO: 2 for the preparation of a medicament for treatment of muscular dystrophy or chronic muscle wasting in a subject in need thereof, wherein the subject has a mutation in the ANO5 gene.

CLAIMS

What is claimed:

1. Use of a recombinant AAV vector comprising 1) a nucleic acid sequence is at least 85% identical to the nucleic acid sequence of SEQ ID NO:1, 2) a nucleotide that
5 hybridizes under stringent condition to the nucleotide sequence of SEQ ID NO: 1 or 3) a nucleic acid sequence encoding a protein that is at least 85% identical to the amino acid sequence of SEQ ID NO: 2 for the preparation of a medicament for treatment of muscular dystrophy or chronic muscle wasting in a subject in need thereof, wherein the subject has a mutation in the ANO5 gene.
- 10 2. The use of claim 1 wherein the recombinant AAV comprises a polynucleotide sequence of SEQ ID NO: 1.
3. The use of any one of claims 1 or 2 wherein the recombinant AAV vector comprises a polynucleotide operably linked to a muscle-specific control element.
4. The use of any one of claims 1 to 3 wherein the subject is suffering from
15 muscular dystrophy.
5. The use of any one of claims 1-4 wherein the medicament is formulated for intramuscular or intravenous injection.
6. The use of any one of the previous claims, wherein the nucleic acid sequence encodes a protein that is at least 95% identical to the amino acid sequence of SEQ ID
20 NO: 2.
7. The use of any one of the previous claims, wherein the nucleic acid sequence encodes a protein comprising the amino acid sequence of SEQ ID NO: 2.
8. The use of any one of the previous claims, wherein the muscular dystrophy is limb girdle muscular dystrophy 2L (LGMD2L) or Miyoshi myopathy dystrophy 3
25 (MMD3).