

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
5 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 a polynucleotide sequence of SEQ ID NO: 1 or a nucleic acid sequence encoding an ANO5 protein comprising the amino acid sequence of SEQ ID
10 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 a polynucleotide sequence of SEQ ID NO: 1 or a nucleic acid sequence encoding an ANO5 protein comprising 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.
2. The use of claim 1 wherein the recombinant AAV vector comprises a polynucleotide operably linked to a muscle-specific control element.
3. The use of any one of claims 1 or 2 wherein the subject is suffering from muscular dystrophy.
4. The use of any one of claims 1-3 wherein the medicament is formulated for intramuscular or intravenous injection.
5. 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.
6. 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 (MMD3).