CLAIMS

1. An isolated and purified nucleotide sequence, comprising:

a micro-dystrophin gene encoding a protein comprising:

an amino-terminal actin-binding domain;

a β-dystroglycan binding domain; and

a spectrin-like repeat domain, consisting of five spectrin-like repeats, including spectrin-like repeat 1 (SR1), spectrin-like repeat 16 (SR16), spectrin-like repeat 17 (SR17), spectrin-like repeat 23 (SR23), and spectrin-like repeat 24 (SR24);

in which the micro-dystrophin gene is operatively linked to a regulatory cassette.

2. An isolated and purified nucleotide sequence according to Claim 1, in which the protein encoded by the micro-dystrophin gene further comprises at least a portion of a hinge domain.

3. An isolated and purified nucleotide sequence according to Claim 2, in which the hinge domain is selected from at least one of a Hinge 1 domain, a Hinge 2 domain, a Hinge 3 domain, a Hinge 4 domain, and a hinge-like domain.

4. An isolated and purified nucleotide sequence according to Claim 1, in which the regulatory cassette is selected from the group consisting of a CK8 promoter and a cardiac troponin T (cTnT) promoter.

5. An isolated and purified nucleotide sequence according to Claim 1, in which the regulatory cassette is a CK8 promoter, and in which the CK8 promoter has at least 80% sequence identity to the nucleic acid sequence of SEQ ID NO: 19.

6. An isolated and purified nucleotide sequence according to Claim 1, in which the regulatory cassette is a cTnT promoter, and in which the cTnT promoter has at least 80% sequence identity to the nucleic acid sequence of SEQ ID NO: 1.

7. An isolated and purified nucleotide sequence according to Claim 1, in which the micro-dystrophin gene encodes a protein comprising:

at least one of spectrin-like repeat 1 directly coupled to spectrin-like repeat 16, spectrin-like repeat 17 directly coupled to spectrin-like repeat 23, and spectrin-like repeat 23 directly coupled to spectrin-like repeat 24.

8. An isolated and purified nucleotide sequence according to Claim 3, which comprises a Hinge 1 domain (H1) and a Hinge 4 domain (H4).

9. A pharmaceutical composition, comprising:

an isolated and purified nucleotide sequence according to Claim 1; and

a delivery vehicle.

10. A pharmaceutical composition according to Claim 9, in which the delivery vehicle comprises an adeno-associated virus (AAV) vector or a recombinant adeno-associated virus (rAAV) vector.

11. A pharmaceutical composition according to Claim 10, in which delivery vehicle comprises an adeno-associated virus (AAV) vector which consists of AAV6, AAV8, or AAV9.

12. A pharmaceutical composition according to Claim 10, in which the delivery vehicle comprises a recombinant adeno-associated virus (rAAV) vector which consists of rAAV6, rAAV8, rAAV9 or rAAV2/6.

13. A pharmaceutical composition according to Claim 10, in which the delivery vehicle comprises an adeno-associated virus (AAV) vector which is serotype AAV9.

14. A pharmaceutical composition according to Claim 10, in which the delivery vehicle comprises a recombinant adeno-associated virus (rAAV) vector which is rAAV9.