CLAIMS

1. A recombinant adeno-associated vector (rAAV) comprising a nucleic acid sequence comprising a transgene encoding alphaLNNdDeltaG2short.

2. A recombinant AAV according to Claim 1, in which the alphaLNNdDeltaG2short comprises SEQ ID NO: 1 or SEQ ID NO: 24.

3. A recombinant AAV according to Claim 1, in which the AAV is AAV8 or AAV-DJ.

4. A recombinant AAV according to Claim 1, further comprising a CMV promoter, preferably in which the CMV promoter comprises SEQ ID NO: 12.

5. A recombinant AAV according to Claim 1, in which the recombinant vector further comprises inverted terminal repeats (ITRs), preferably in which the inverted terminal repeat (ITR) is a 5' ITR comprising SEQ ID NO: 11 or a 3' ITR comprising SEQ ID NO: 16.

6. A recombinant adeno-associated vector (rAAV) comprising a nucleic acid sequence comprising a transgene encoding alphaLNNdDeltaG2Propeller, in which the nucleic acid sequence comprises either: (a) SEQ ID NOs: 25, 29, 31, 33, 35, 41, 45 and 55; (b) SEQ ID NOs: 25, 29, 31, 33, 35, 41, 47 and 55; or (c) SEQ ID NOs: 25, 29, 31, 33, 35, 41, 51 and 55.

7. A recombinant adeno-associated vector (rAAV) comprising a nucleic acid sequence comprising a transgene encoding alphaLNNdDeltaG2Propeller-2, in which the nucleic acid sequence comprises SEQ ID NOs: 25, 29, 31, 33, 41, 43, 45 and 55.

8. A recombinant adeno-associated vector (rAAV) comprising a nucleic acid sequence comprising a transgene encoding betaLNNdDeltaG2short, in which the nucleic acid sequence comprises SEQ ID NOs: 59, 63, 67, 71, 75, 79, 49, 51, 53, 55 and 57.

9. A recombinant adeno-associated vector (rAAV) comprising a nucleic acid sequence comprising a transgene encoding gammaLNNdDeltaG2short, in which the nucleic acid sequence comprises SEQ ID NOs: 83, 87, 91, 95, 99,103, 49, 51, 53, 55 and 57.

10. A pharmaceutical composition comprising a recombinant AAV according to any of Claims 1, 2 and 6 to 9 and a pharmaceutical carrier.

11. A kit comprising a container housing comprising a composition according to Claim 10.

12. A recombinant AAV according to any of Claims 1, 2 and 6 to 9 for use in a method of restoring laminin polymerization expression and basement membrane assembly in a subject, in which an effective amount of the recombinant AAV vector is to be administered to the subject.

13. A recombinant AAV according to Claim 1 for use in a method of treating laminin α-2 deficiency syndrome in a subject in need thereof, in which an effective amount of the recombinant AAV vector is to be administered to the subject.

14. A recombinant AAV according to Claim 1 for use in a method of alleviating in a subject at least one of the symptoms associated with laminin deficiencies selected from the group consisting of laminin-deficient muscular dystrophies and laminin α2-deficient muscular dystrophy, in which an effective amount of the recombinant AAV vector is to be administered to the subject.

15. A recombinant AAV according to Claim 1 for use in a method of alleviating in a subject at least one of the symptoms associated with laminin α2-deficiencies selected from the group consisting of muscle degeneration, regeneration, chronic inflammation, fibrosis, white matter brain anomalies, reduced peripheral nerve conduction, seizures, moderate mental retardation, and respiratory failure, in which an effective amount of the recombinant AAV vector is to be administered to the subject.

16. A recombinant AAV for use according to any of Claim 13, 14 or 15, in which the alphaLNNdDeltaG2short comprises SEQ ID NO: 1 or SEQ ID NO: 24.

17. A recombinant AAV for use according to any of Claims 13, 14 or 15, in which the AAV is AAV8 or AAV.DJ.

18. A recombinant AAV for use according to any of Claims 13, 14 or 15, in which the recombinant AAV further comprises a CMV promoter, preferably in which the in which the CMV promoter comprises SEQ ID NO: 12.

19. A recombinant AAV for use according to any of Claims 13, 14 or 15, in which the recombinant vector further comprises inverted terminal repeats (ITRs), preferably in which

the inverted terminal repeat (ITR) is a 5' ITR comprising SEQ ID NO: 11 or a 3' ITR comprising SEQ ID NO: 16.

20. A recombinant AAV for use according to any of Claims 13, 14 or 15, in which the recombinant AAV is comprised within a pharmaceutical composition further comprising a pharmaceutical carrier.

CLAIMS

1. A recombinant adeno-associated virus vector (rAAV) comprising a nucleic acid sequence comprising a transgene encoding alpha laminin N-terminal domain delta G2 short linking protein (alphaLNNdDeltaG2short), in which the alphaLNNdDeltaG2short comprises SEQ ID NO: 1 or SEQ ID NO: 24.

2. An rAAV according to Claim 1, in which the AAV is AAV8 or AAV-DJ.

3. An rAAV according to Claim 1, further comprising a CMV promoter, preferably in which the CMV promoter comprises SEQ ID NO: 12.

4. An rAAV according to Claim 1, in which the rAAV further comprises inverted terminal repeats (ITRs), preferably in which the inverted terminal repeat (ITR) is a 5' ITR comprising SEQ ID NO: 11 or a 3' ITR comprising SEQ ID NO: 16.

5. A pharmaceutical composition comprising an rAAV according to Claim 1 and a pharmaceutical carrier.

6. A kit comprising a container housing comprising a composition according to Claim 5.

7. An rAAV according to Claim 1 for use in a method of restoring laminin polymerization expression and basement membrane assembly in a subject, in which an effective amount of the rAAV is to be administered to the subject.

8. An rAAV according to Claim 1 for use in a method of treating laminin α-2 deficiency syndrome in a subject in need thereof, in which an effective amount of the rAAV is to be administered to the subject.

9. An rAAV according to Claim 1 for use in a method of alleviating in a subject at least one of the symptoms associated with laminin deficiencies selected from the group consisting of laminin-deficient muscular dystrophies and laminin α2-deficient muscular dystrophy, in which an effective amount of the rAAV is to be administered to the subject.

10. An rAAV according to Claim 1 for use in a method of alleviating in a subject at least one of the symptoms associated with laminin α2-deficiencies selected from the group consisting of muscle degeneration, regeneration, chronic inflammation, fibrosis, white matter

brain anomalies, reduced peripheral nerve conduction, seizures, moderate mental retardation, and respiratory failure, in which an effective amount of the rAAV is to be administered to the subject.

11. An rAAV for use according to any of Claims 8, 9 or 10, in which the AAV is AAV8 or AAV-DJ.

12. An rAAV for use according to any of Claims 8, 9 or 10, in which the rAAV further comprises a CMV promoter, preferably in which the CMV promoter comprises SEQ ID NO: 12.

13. An rAAV for use according to any of Claims 8, 9 or 10, in which the rAAV further comprises inverted terminal repeats (ITRs), preferably in which the inverted terminal repeat (ITR) is a 5' ITR comprising SEQ ID NO: 11 or a 3' ITR comprising SEQ ID NO: 16.

14. An rAAV for use according to any of Claims 8, 9 or 10, in which the rAAV is comprised within a pharmaceutical composition further comprising a pharmaceutical carrier.