

CLAIMS

1. A recombinant adeno-associated vector (rAAV) comprising a transgene encoding a variant alphaLNNd wherein the variant alphaLNNd comprises a nucleic acid sequence comprising SEQ ID NO: 171, SEQ ID NO: 173, SEQ ID NO: 175, SEQ ID NO: 179, SEQ ID NO: 1 or SEQ ID NO: 24.
2. The recombinant AAV of claim 1, wherein the AAV is AAV8, AAV-9 or AAV-DJ.
3. The recombinant AAV of claim 1, further comprising a CMV promoter.
4. The recombinant AAV of claim 3, wherein the CMV promoter comprises SEQ ID NO: 12.
5. The recombinant AAV of claim 1, wherein the recombinant vector further comprises inverted terminal repeats (ITRs).
6. The recombinant AAV of claim 5, wherein the inverted terminal repeat (ITR) is a 5' ITR comprising SEQ ID NO: 11.
7. The recombinant AAV of claim 5, wherein the inverted terminal repeat (ITR) is a 3' ITR comprising SEQ ID NO: 16.
8. A pharmaceutical composition comprising the recombinant AAV of claims 1 and a pharmaceutical carrier.
9. A method of restoring laminin polymerization expression and basement membrane assembly in a subject, comprising administering to the subject an effective amount of the recombinant AAV vector of claims 1.
10. A method of treating laminin α -2 deficiency syndrome in a subject in need thereof, wherein the method comprises administering to the subject an effective amount of the recombinant AAV vector of claim 1.
11. 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 α 2-deficient muscular dystrophy, wherein the method comprises administering to the subject an effective amount of the recombinant AAV vector of claim 1.
12. A method of alleviating in a subject at least one of the symptoms associated with laminin α 2-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, wherein the method comprises administering to the subject an effective amount of the recombinant AAV vector of claim 1.

13. The method of claim 10, 11, or 12, wherein the AAV is AAV8, AAV-9 or AAV-DJ.
14. The method of claim 10, 11, or 12, wherein the recombinant AAV further comprises a CMV promoter.
15. The method of claim 10, 11, or 12, wherein the recombinant vector further comprises inverted terminal repeats (ITRs).