

CLAIMS

~~WHAT IS CLAIMED IS:~~

1. A nucleic acid molecule that binds or is complementary to a polynucleotide encoding exon 44 of the DMD gene, wherein the polynucleotide encoding exon 44 comprises or consists of the nucleotide sequence set out in SEQ ID NO: 1 or 2 or encodes the amino acid sequence set out in SEQ ID NO: 3.
2. The nucleic acid molecule of claim 1 that binds or is complementary to at least one of the nucleotide sequences set out in SEQ ID NO: 4, 5, 6, 7, 32, 33, 34, or 35.
3. The nucleic acid molecule of claim 1 or 2 comprising or consisting of a nucleotide sequence having at least 80%, at least 85%, at least 90%, at least 95%, at least 96%, at least 97%, at least 98%, or at least 99% identity to the nucleotide sequence set out in SEQ ID NO: 4, 5, 6, 7, 8, 9, 10, 11, 12, 13, 14, 15, 32, 33, 34, or 35.
4. The nucleic acid molecule of any one of claims 1-3 comprising or consisting of a nucleotide sequence having at least 80%, at least 85%, at least 90%, at least 95%, at least 96%, at least 97%, at least 98%, or at least 99% identity to the nucleotide sequence set out in SEQ ID NO: 16, 17, 18, 19, 20, 21, 22, 23, 24, 25, 26, 27, or 28.
5. The nucleic acid molecule of claim 1, 2, or 3 comprising or consisting of the nucleotide sequence set out in SEQ ID NO: 4, 5, 6, 7, 8, 9, 10, 11, 12, 13, 14, 15, 32, 33, 34, or 35.
6. The nucleic acid molecule of any one of claims 1-4 comprising or consisting of the nucleotide sequence set out in SEQ ID NO: 16, 17, 18, 19, 20, 21, 22, 23, 24, 25, 26, 27, or 28.
7. A recombinant adeno-associated virus (rAAV) comprising a genome comprising at least one of the nucleic acid molecules of any one of claims 1-6.
8. The rAAV of claim 7 wherein the genome is a self-complementary genome or a single-stranded genome.
9. The rAAV of claim 7 or 8 wherein the rAAV is rAAV-1, rAAV-2, rAAV-3, rAAV-4, rAAV-5, rAAV-6, rAAV-7, rAAV-8, rAAV-9, rAAV-10, rAAV-11, rAAV-12, rAAV-13, rAAV-rh74, or rAAV-anc80.
10. The rAAV of claim of any one of claims 7-9 wherein the genome of the rAAV lacks AAV rep and cap DNA.

11. The rAAV of claim 10 further comprising an AAV-1 capsid, an AAV-2 capsid, an AAV-3 capsid, an AAV-4 capsid, an AAV-5 capsid, an AAV-6 capsid, an AAV-7 capsid, an AAV-8 capsid, an AAV-9 capsid, an AAV-10 capsid, an AAV-11 capsid, an AAV-12 capsid, an AAV-13 capsid, an AAV-rh74 capsid, or an AAV-anc80 capsid.

12. ~~A method for inducing skipping of exon 44 of the DMD gene in a cell, the method comprising providing the cell with the~~ The nucleic acid molecule of any one of claims 1-6 for use in therapy.

13. ~~A method for inducing skipping of exon 44 of the DMD gene in a cell, the method comprising providing the cell with the~~ The rAAV of any one of claims 7-11 for use in therapy.

14. ~~A method~~ Use of at least one of the nucleic acid molecules of any one of claims 1-6 in the manufacture of a medicament for treating, ameliorating, and/or preventing a muscular dystrophy in a subject with a mutation amenable to skipping exon 44 of the DMD gene (DMD exon 44) ~~comprising administering to the subject at least one of the nucleic acid molecules of any one of claims 1-6.~~

15. ~~A method~~ Use of at least one of the rAAV of any one of claims 7-11 in the manufacture of a medicament for treating, ameliorating, and/or preventing a muscular dystrophy in a subject with a mutation amenable to skipping exon 44 of the DMD gene (DMD exon 44) ~~comprising administering to the subject at least one of the rAAV of any one of claims 7-11.~~

16. The ~~method~~ use of claim 14 or 15, wherein the mutation is any mutation involving, surrounding, or affecting DMD exon 44.

17. The ~~method~~ use of claim 16, wherein the mutation is a duplication of DMD exon 44, a deletion of exon 43 or 45, or a deletion of exons 45-56.

18. The ~~method~~ use of any one of claims 14-17, wherein the ~~administering medicament~~ results in increased expression of dystrophin protein in the subject.

19. The ~~method~~ use of any one of claims 14-17, wherein the ~~administering medicament~~ inhibits the progression of dystrophic pathology in the subject.

20. The ~~method~~ use of any one of claims 14-17, wherein the ~~administering medicament~~ improves muscle function in the subject.

21. The ~~method~~ use of claim 20 wherein the improvement in muscle function is an improvement in muscle strength.

22. The ~~method~~use of claim 20 wherein the improvement in muscle function is an improvement in stability in standing and walking.

23. ~~Use of at least one nucleic acid molecule of any one of claims 1-6 in treating, ameliorating, and/or preventing a muscular dystrophy in a subject with a mutation amenable to skipping exon 44 of the DMD gene (DMD exon 44).~~

24. ~~Use of at least one rAAV of any one of claims 7-11 in treating, ameliorating, and/or preventing a muscular dystrophy in a subject with a mutation amenable to skipping exon 44 of the DMD gene (DMD exon 44).~~