CLAIMS

1. A nucleic acid molecule that binds or is complementary to

(a) a target DNA nucleotide sequence encoding exon 44 of the DMD gene and its surrounding intronic sequence, wherein the target nucleotide sequence consists of the nucleotide sequence of SEQ ID NO: 4; or

(b) a target mRNA nucleotide sequence of exon 44 of the DMD gene, wherein the target mRNA nucleotide sequence consists of the nucleotide sequence of SEQ ID NO: 32.

2. The nucleic acid molecule of claim 1 comprising or consisting of

(a) an antisense sequence of the target DNA nucleotide sequence encoding exon 44 of the DMD gene, wherein the antisense sequence comprises or consists 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% sequence identity to the nucleotide sequence of SEQ ID NO: 8;

(b) an antisense sequence of the target DNA nucleotide sequence encoding exon 44 of the DMD gene, wherein the antisense sequence comprises or consists of the nucleotide sequence of SEQ ID NO: 8;

(c) an antisense sequence of the mRNA nucleotide sequence of exon 44 of the DMD gene, wherein the antisense sequence of the mRNA comprises or consists 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% sequence identity to the nucleotide sequence of SEQ ID NO: 12; or

(d) an antisense sequence of the mRNA nucleotide sequence of exon 44 of the DMD gene, wherein the antisense sequence of the mRNA comprises or consists of the nucleotide sequence of SEQ ID NO: 12.

3. The nucleic acid molecule of claim 1 or 2 comprising or consisting of

(a) a nucleotide sequence having at least 96%, at least 97%, at least 98%, or at least 99% sequence identity to the nucleotide sequence of SEQ ID NO: 16 or 20; or

(b) the nucleotide sequence of SEQ ID NO: 16 or 20.

4. A recombinant adeno-associated virus (rAAV) vector comprising a genome comprising at least one of the nucleic acid molecules of any one of claims 1-3.

5. The rAAV vector of claim 4 wherein the genome is a self-complementary genome or a single-stranded genome.

6. The rAAV vector of claim 4 or 5 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.

7. The rAAV vector of claim of any one of claims 4-6 wherein the genome of the rAAV lacks AAV rep and cap DNA.

8. The rAAV vector of claim 7 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.

9. The nucleic acid molecule of any one of claims 1-3 for use in therapy.

10. The rAAV vector of any one of claims 4-8 for use in therapy.

11. Use of at least one of the nucleic acid molecules of any one of claims 1-3 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).

12. Use of at least one of the rAAV of any one of claims 4-8 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).

13. The use of claim 11 or 12, wherein the mutation is any mutation involving, surrounding, or affecting DMD exon 44.

14. The use of claim 13, wherein the mutation is a duplication of DMD exon 44, a deletion of exon 43 or 45, or a deletion of exons 45-56.

15. The use of any one of claims 11-14, wherein the medicament results in increased expression of dystrophin protein in the subject.

16. The use of any one of claims 11-14, wherein the medicament inhibits the progression of dystrophic pathology in the subject.

17. The use of any one of claims 11-14, wherein the medicament improves muscle function in the subject.

18. The use of claim 17 wherein the improvement in muscle function is an improvement in muscle strength.

19. The use of claim 17 wherein the improvement in muscle function is an improvement in stability in standing and walking.