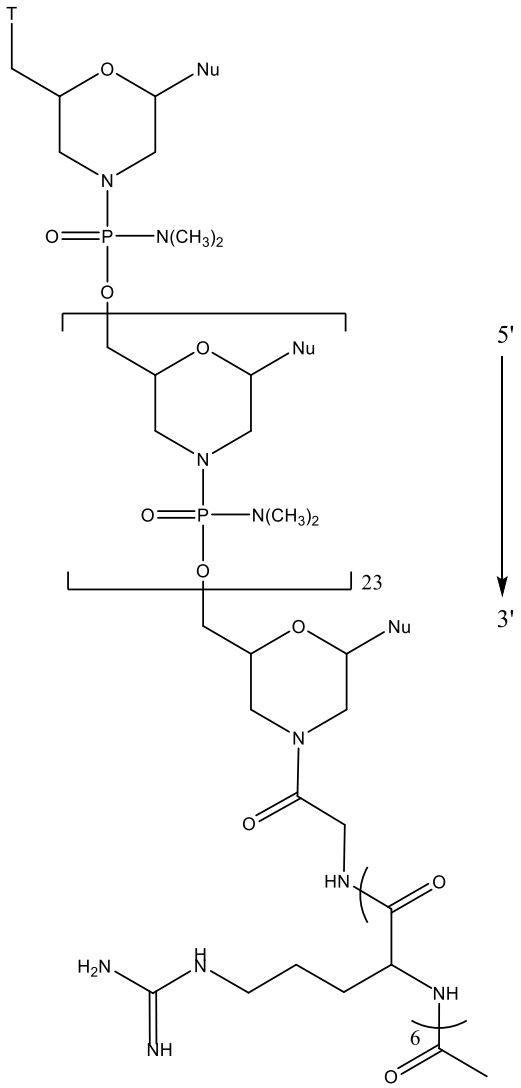
CLAIMS

We claim:

1. An antisense oligomer conjugate of Formula (I):

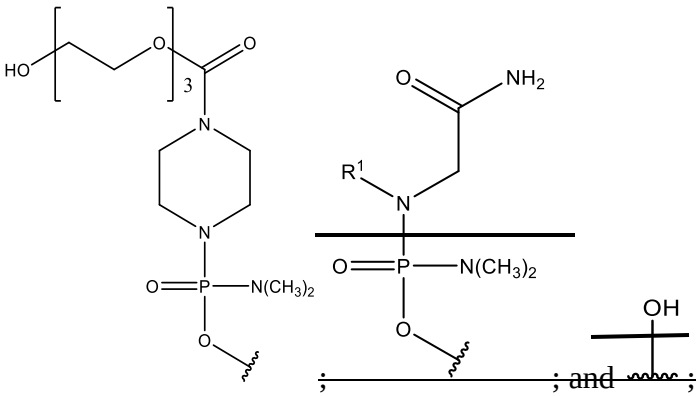


(I)

or a pharmaceutically acceptable salt thereof, wherein:

each Nu is a nucleobase which taken together form a targeting sequence; and

T is a moiety ~~selected from~~ of formula:



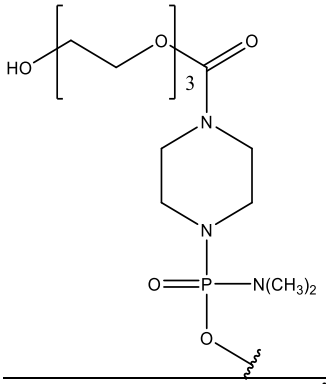
~~;~~ ; ~~and~~ ;

R^1 is C_1-C_6 alkyl;

wherein the targeting sequence is ~~complementary to an exon 52 annealing site in the dystrophin pre-mRNA designated as H52A(-01+24)~~ SEQ ID NO: 1 (5'-CTGTTCCAAATCCTGCATTGTTGCC-3').

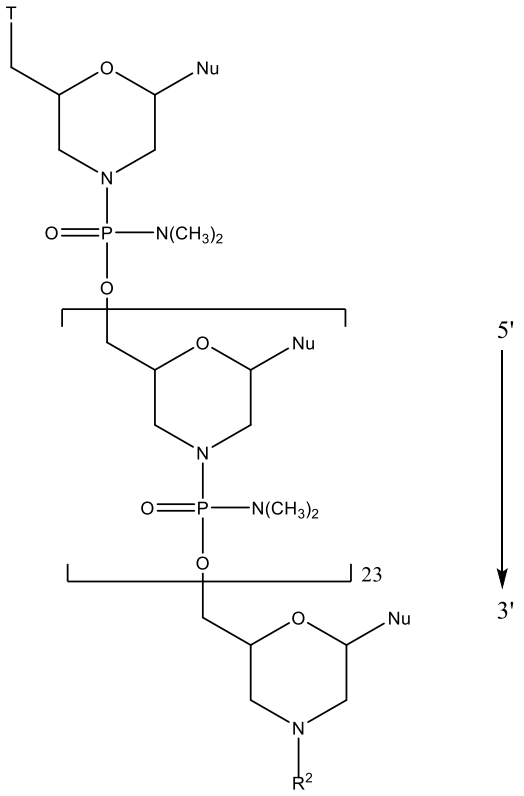
~~2. The antisense oligomer conjugate of claim 1, wherein each Nu is independently selected from cytosine (C), guanine (G), thymine (T), adenine (A), 5-methylcytosine (5mC), uracil (U), and hypoxanthine (I).~~

~~3. The antisense oligomer conjugate of claim 1, wherein T is~~



~~, and the targeting sequence is SEQ ID NO: 1 (5'-CTGTTCCAAATCCTGCATTGTTGCC-3').~~

~~4~~2. An antisense oligomer of Formula (V):

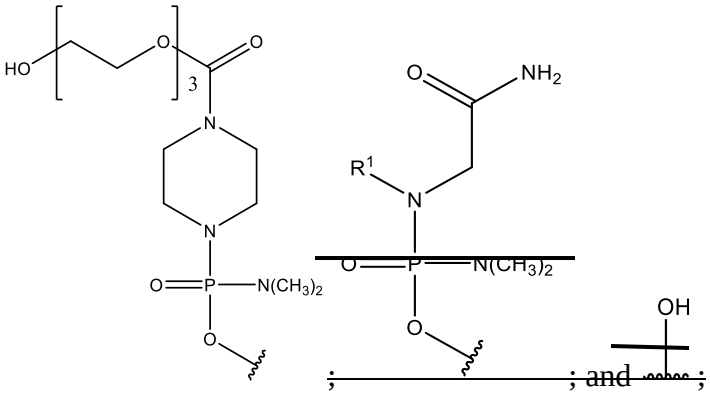


(V)

or a pharmaceutically acceptable salt thereof, wherein:

each Nu is a nucleobase which taken together form a targeting sequence; and

T is <u>of formula</u> ~~a moiety selected from~~:



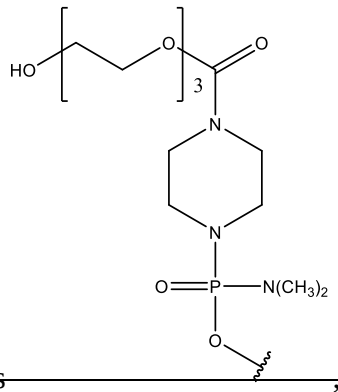
; ; and ;

R^1 is C_1-C_6 alkyl; and

R^2 is selected from H or acetyl,

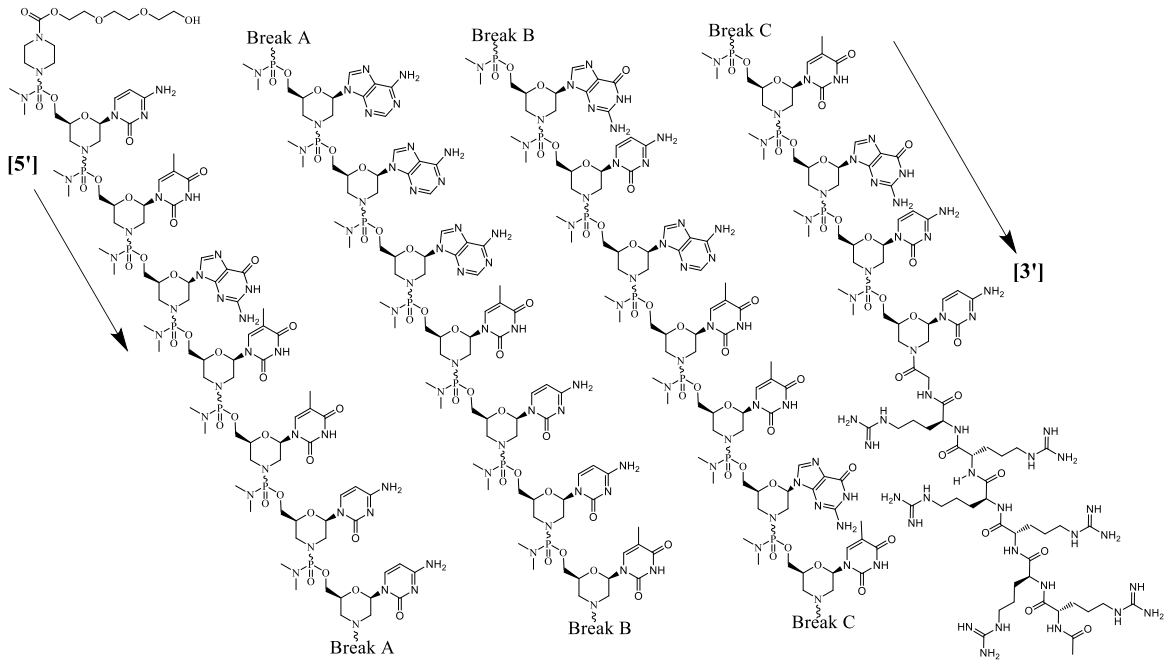
wherein the targeting sequence is SEQ ID NO: 1 (5’-CTGTTCCAAATCCTGCATTGTTGCC-3’). ~~complementary to an exon 52 annealing site in the dystrophin pre-mRNA designated as H52A(-01+24).~~

~~5. The antisense oligomer of claim 4, wherein each Nu is independently selected from cytosine (C), guanine (G), thymine (T), adenine (A), 5-methylcytosine (5mC), uracil (U), and hypoxanthine (I).~~



~~6. The antisense oligomer of claim 4, wherein T is , and the targeting sequence is SEQ ID NO: 1 (5’-CTGTTCCAAATCCTGCATTGTTGCC-3’).~~

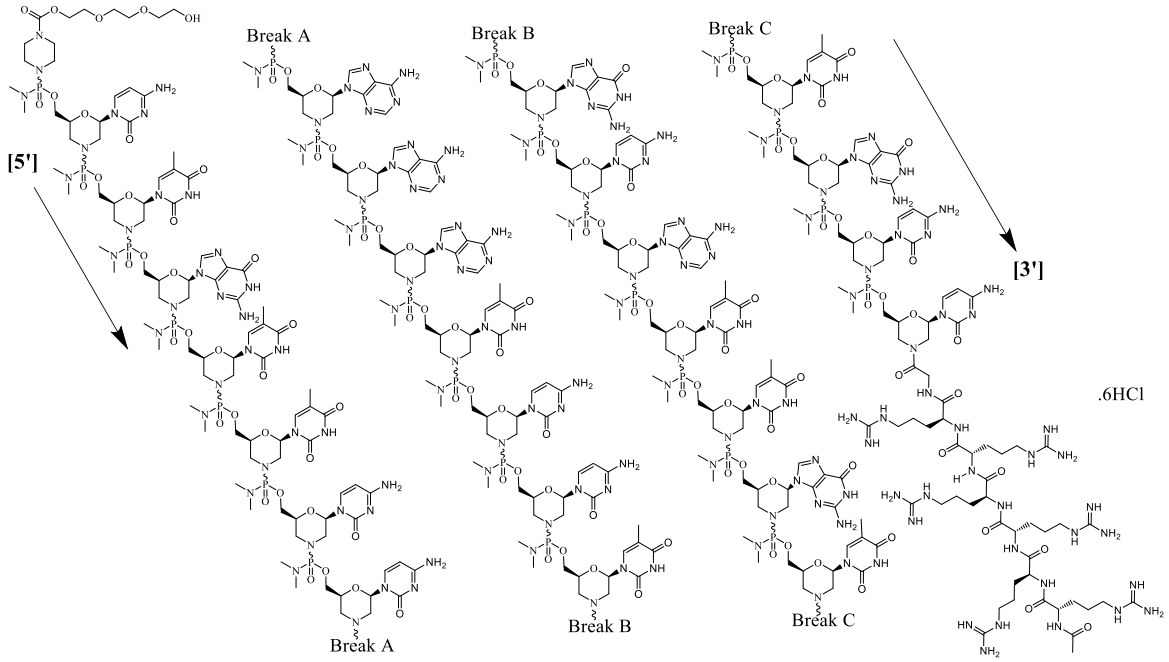
~~7~~3. An antisense oligomer conjugate of Formula (IV):



(IV)

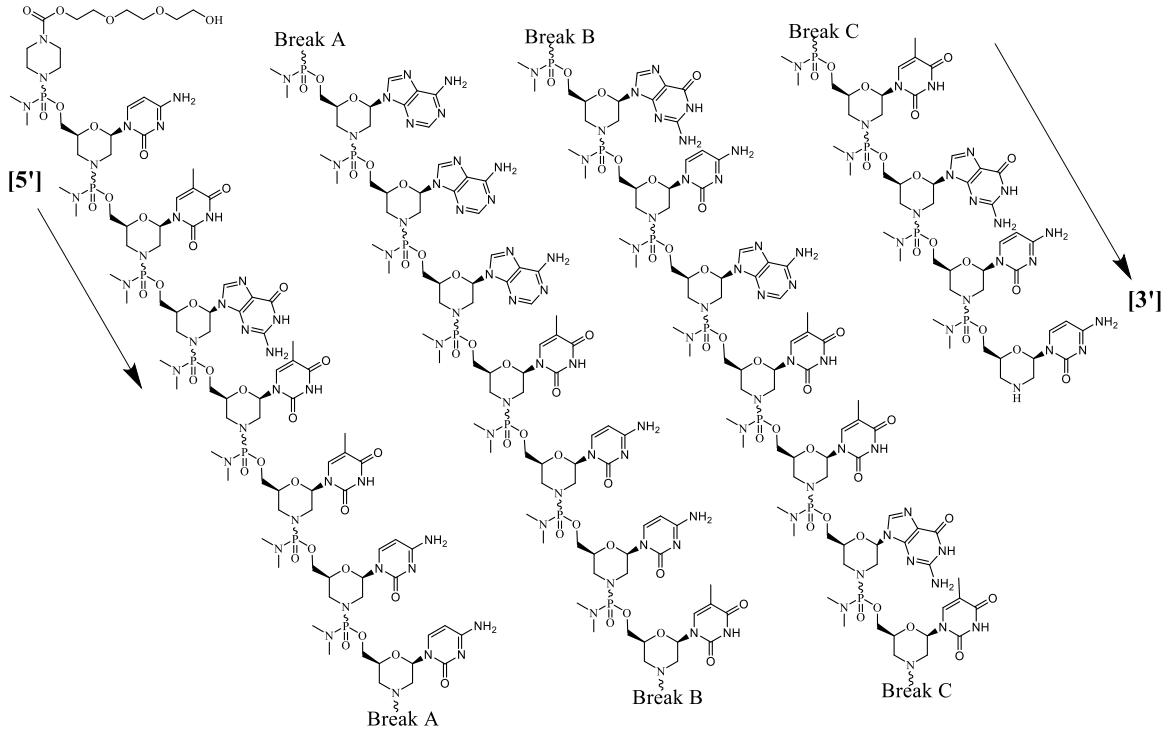
or a pharmaceutically acceptable salt thereof.

~~8~~4. The antisense oligomer conjugate of claim ~~7~~3, wherein the antisense oligomer is of Formula (IVA):



(IVA).

~~9~~5. An antisense oligomer of Formula (VIIA):



(VIIA)

or a pharmaceutically acceptable salt thereof.

~~10. A pharmaceutical composition, comprising an antisense oligomer conjugate of any one of claims 1 to 3, 7 or 8, or a pharmaceutically acceptable salt thereof, and a pharmaceutically acceptable carrier.~~

~~11. Use of the antisense oligomer conjugate of any one of claims 1 to 3, 7 or 8 in the manufacture of a medicament for the treatment of Duchenne muscular dystrophy (DMD) in a subject in need thereof wherein the subject has a mutation of the dystrophin gene that is amenable to exon 52 skipping.~~

~~12. The use of claim 11, wherein the antisense oligomer conjugate is administered weekly, biweekly, every third week, or monthly.~~

13. The use of any one of claims 11 or 12, wherein the antisense oligomer conjugate is administered at a dose of about 30 mg/kg, about 40 mg/kg, about 60 mg/kg, about 80 mg/kg, or about 160 mg/kg.

14. A method of restoring an mRNA reading frame to induce dystrophin production in a subject having a mutation of the dystrophin gene that is amenable to exon 52 skipping, the method comprising administering to the subject the antisense oligomer conjugate of any one of claims 1 to 3, 7 or 8.

15. The method of claim 14, wherein the antisense oligomer conjugate is to be administered weekly, biweekly, every third week, or monthly.

16. The method of any one of claims 14 or 15, wherein the antisense oligomer conjugate is administered at a dose of about 30 mg/kg, about 40 mg/kg, about 60 mg/kg, about 80 mg/kg, or about 160 mg/kg.

17. A method of restoring an mRNA reading frame to induce dystrophin production in a subject having a mutation of the dystrophin gene that is amenable to exon 52 skipping, the method comprising administering to the subject the pharmaceutical composition of claim 10.

18. A method of excluding exon 52 from dystrophin pre-mRNA during mRNA processing in a subject having a mutation of the dystrophin gene that is amenable to exon 52 skipping, the method comprising administering to the subject the pharmaceutical composition of claim 10.

19. A method of binding exon 52 of dystrophin pre-mRNA in a subject having a mutation of the dystrophin gene that is amenable to exon 52 skipping, the method comprising administering to the subject the pharmaceutical composition of claim 10.

20. A pharmaceutical composition, comprising an antisense oligomer of any one of claims 4-6 or 9, or a pharmaceutically acceptable salt thereof, and a pharmaceutically acceptable carrier.

21. Use of the antisense oligomer of any one of claims 4-6 or 9 in the manufacture of a medicament for the treatment of Duchenne muscular dystrophy (DMD) in a subject in need

thereof wherein the subject has a mutation of the dystrophin gene that is amenable to exon 52 skipping.

22. A method of restoring an mRNA reading frame to induce dystrophin production in a subject having a mutation of the dystrophin gene that is amenable to exon 52 skipping, the method comprising administering to the subject the antisense oligomer of any one of claims 4-6 or 9.

23. A method of restoring an mRNA reading frame to induce dystrophin production in a subject having a mutation of the dystrophin gene that is amenable to exon 52 skipping, the method comprising administering to the subject the pharmaceutical composition of claim 21.

24. A method of excluding exon 52 from dystrophin pre-mRNA during mRNA processing in a subject having a mutation of the dystrophin gene that is amenable to exon 52 skipping, the method comprising administering to the subject the pharmaceutical composition of claim 21.

26. A method of binding exon 52 of dystrophin pre-mRNA in a subject having a mutation of the dystrophin gene that is amenable to exon 52 skipping, the method comprising administering to the subject the pharmaceutical composition of claim 21.

27. The antisense oligomer of any one of claims 1 to 3, 7 or 8, or the pharmaceutical composition of claim 10 for use in the treatment of Duchenne muscular dystrophy (DMD) in a subject in need thereof wherein the subject has a mutation of the dystrophin gene that is amenable to exon 52 skipping.

28. The antisense oligomer of any one of claims 4-6 or 9, or the pharmaceutical composition of claim 20 for use in the treatment of Duchenne muscular dystrophy (DMD) in a subject in need thereof wherein the subject has a mutation of the dystrophin gene that is amenable to exon 52 skipping.