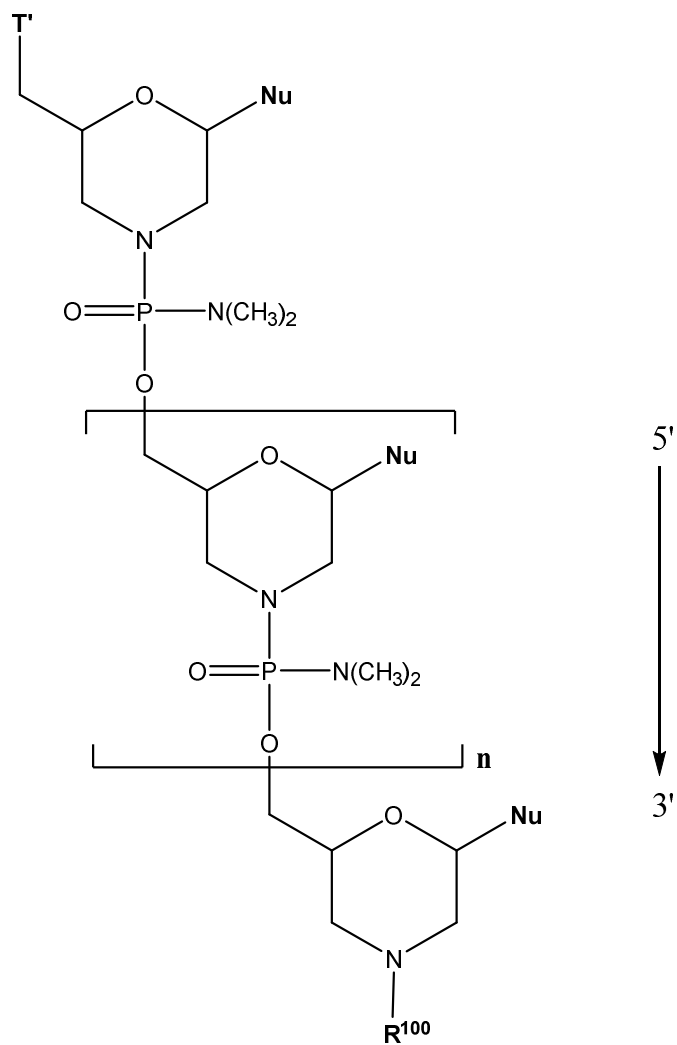


CLAIMS

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



(I),

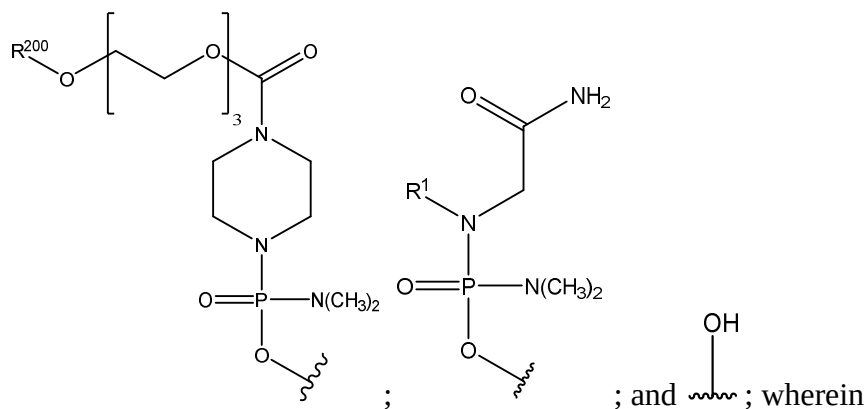
or a pharmaceutically acceptable salt thereof,

wherein:

n is 1-40;

each **Nu** is a nucleobase, which, taken together, form a targeting sequence complementary to an exon annealing site in the dystrophin pre-mRNA;

T' is a moiety selected from:

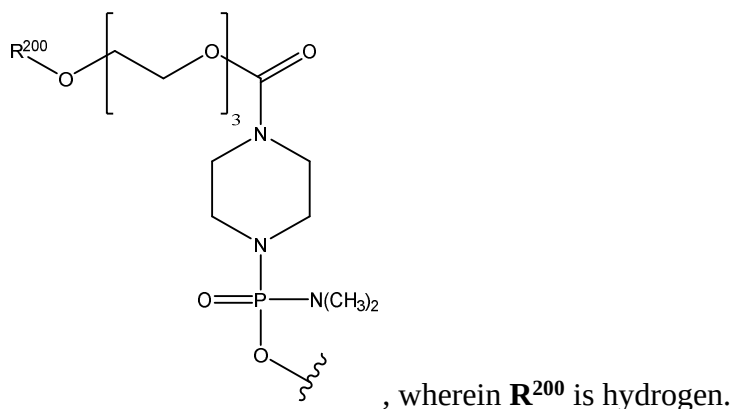


R¹⁰⁰ is selected from the group consisting of RRRRRG-, RRRRG-, RRRG-, RRG-, RG-, and G-, wherein R is arginine and G is glycine,

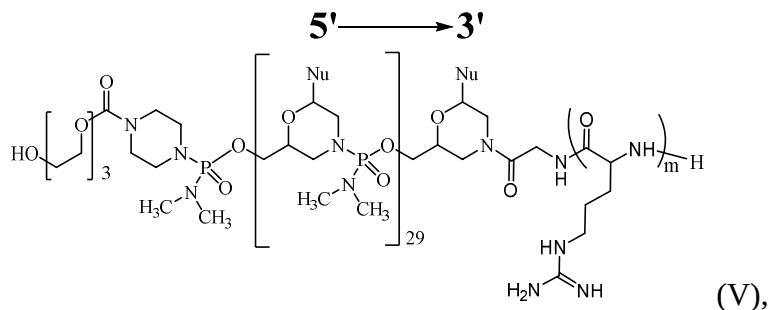
R²⁰⁰ is hydrogen, and

R¹ is C₁-C₆ alkyl.

2. The antisense oligomer conjugate of claim 1, or a pharmaceutically acceptable salt thereof, wherein the targeting sequence is complementary to an exon 51 annealing site in the dystrophin pre-mRNA designated as H51A(+66+95).
3. The antisense oligomer conjugate of claim 1, or a pharmaceutically acceptable salt thereof, wherein the targeting sequence is complementary to an exon 45 annealing site in the dystrophin pre-mRNA designated as H45A(-03+19).
4. The antisense oligomer conjugate of claim 1, or a pharmaceutically acceptable salt thereof, wherein the targeting sequence is complementary to an exon 53 annealing site in the dystrophin pre-mRNA designated as H53A(+36+60).
5. The antisense oligomer conjugate of any one of claims 1-4, or a pharmaceutically acceptable salt thereof, 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 conjugate of any one of claims 1-5, or a pharmaceutically acceptable salt thereof, wherein **T'** is a moiety:

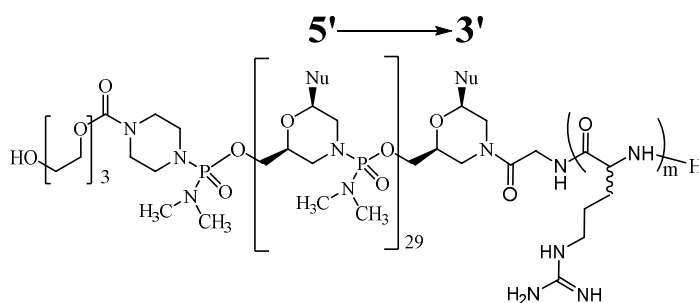


7. The antisense oligomer conjugate of any one of claims 1-6, or a pharmaceutically acceptable salt thereof, wherein **R**¹⁰⁰ is RRRRRG-.
8. The antisense oligomer conjugate of any one of claims 1-6, or a pharmaceutically acceptable salt thereof, wherein **R**¹⁰⁰ is RRRRG-.
9. The antisense oligomer conjugate of any one of claims 1-6, or a pharmaceutically acceptable salt thereof, wherein **R**¹⁰⁰ is RRRG-.
10. The antisense oligomer conjugate of any one of claims 1-6, or a pharmaceutically acceptable salt thereof, wherein **R**¹⁰⁰ is RRG-.
11. The antisense oligomer conjugate of any one of claims 1-6, or a pharmaceutically acceptable salt thereof, wherein **R**¹⁰⁰ is RG-.
12. The antisense oligomer conjugate of any one of claims 1-6, or a pharmaceutically acceptable salt thereof, wherein **R**¹⁰⁰ is G-.
13. The antisense oligomer conjugate of any one of claims 1-12, having the Formula (V):



or a pharmaceutically acceptable salt thereof, wherein each Nu is a nucleobase, which, taken together, form a targeting sequence that is complementary to an exon annealing site in the dystrophin pre-mRNA, and m is 0, 1, 2, 3, 4, or 5.

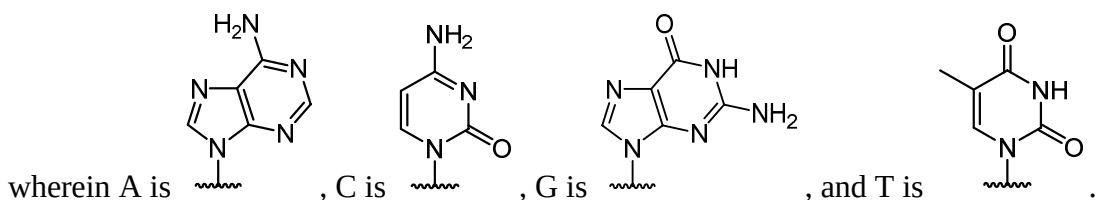
14. The antisense oligomer conjugate of any one of claims 1-13, having the Formula (VA):



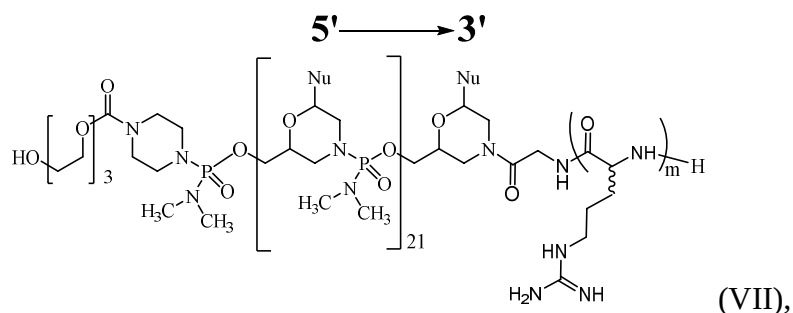
(VA),

or a pharmaceutically acceptable salt thereof, wherein m is 0, 1, 2, 3, 4, or 5, and each Nu from 1 to 30 and 5' to 3' is:

Position No. 5' to 3'	Nu	Position No. 5' to 3'	Nu	Position No. 5' to 3'	Nu	Position No. 5' to 3'	Nu	Position No. 5' to 3'	Nu	Position No. 5' to 3'	Nu
1	C	6	A	11	A	16	A	21	G	26	T
2	T	7	C	12	A	17	G	22	C	27	C
3	C	8	A	13	G	18	A	23	A	28	T
4	C	9	T	14	G	19	T	24	T	29	A
5	A	10	C	15	A	20	G	25	T	30	G

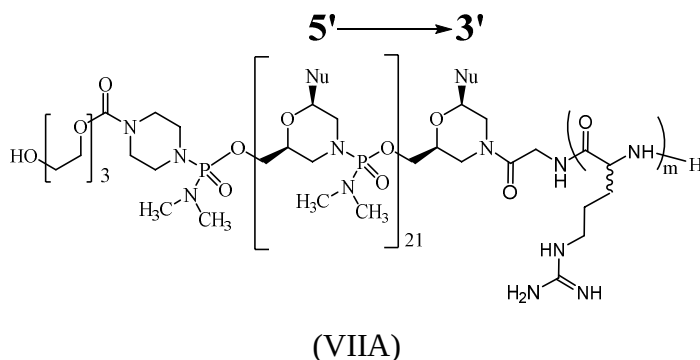


15. The antisense oligomer conjugate of any one of claims 1-12, having the Formula (VII):



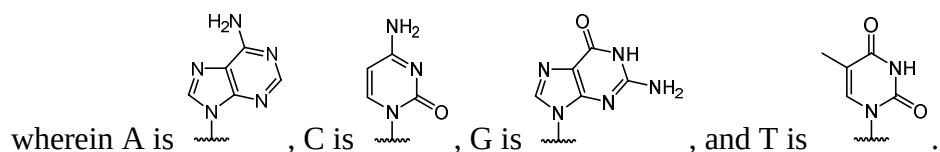
or a pharmaceutically acceptable salt thereof, wherein each Nu is a nucleobase, which, taken together, form a targeting sequence that is complementary to an exon annealing site in the dystrophin pre-mRNA, and m is 0, 1, 2, 3, 4, or 5.

16. The antisense oligomer conjugate of any one of claims 1-12 or 15, having the Formula (VIIA):

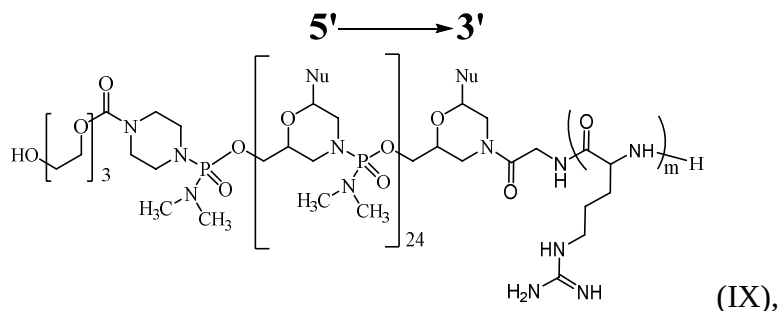


or a pharmaceutically acceptable salt thereof, wherein m is 0, 1, 2, 3, 4, or 5, and each Nu from 1 to 22 and 5' to 3' is:

Position No. 5' to 3'	Nu	Position No. 5' to 3'	Nu	Position No. 5' to 3'	Nu	Position No. 5' to 3'	Nu	Position No. 5' to 3'	Nu
1	C	6	C	11	C	16	G	21	T
2	A	7	C	12	T	17	T	22	G
3	A	8	A	13	G	18	T		
4	T	9	T	14	G	19	C		
5	G	10	C	15	A	20	C		

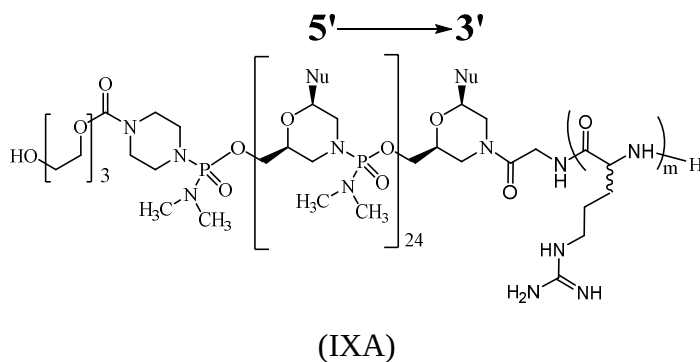


17. The antisense oligomer conjugate of any one of claims 1-12, having the Formula (IX):



or a pharmaceutically acceptable salt thereof, wherein each Nu is a nucleobase, which, taken together, form a targeting sequence that is complementary to an exon annealing site in the dystrophin pre-mRNA, and m is 0, 1, 2, 3, 4, or 5.

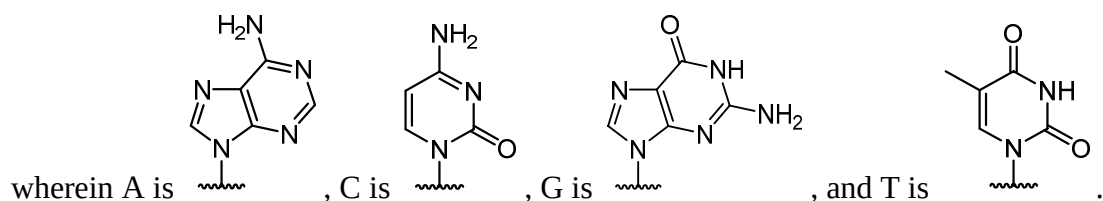
18. The antisense oligomer conjugate of any one of claims 1-12 or 17, having the Formula (IXA):



or a pharmaceutically acceptable salt thereof, wherein m is 0, 1, 2, 3, 4, or 5, and each Nu from 1 to 25 and 5' to 3' is:

Position	Nu	Position	Nu	Position	Nu	Position	Nu	Position	Nu
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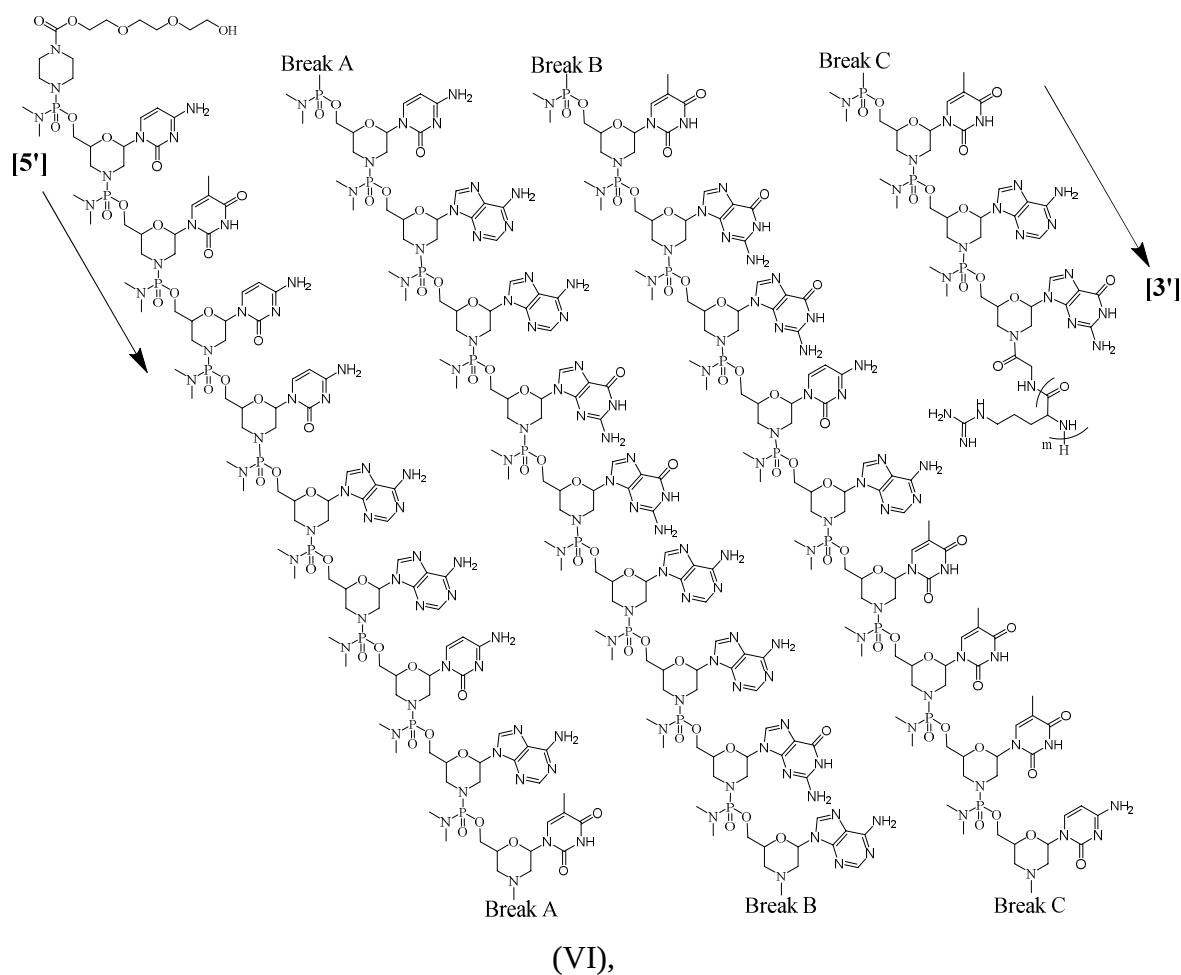
No. 5' to 3'		No. 5' to 3'		No. 5' to 3'		No. 5' to 3'		No. 5' to 3'	
1	G	6	C	11	G	16	G	21	T
2	T	7	T	12	T	17	A	22	G
3	T	8	C	13	T	18	A	23	T
4	G	9	C	14	C	19	G	24	T
5	C	10	G	15	T	20	G	25	C



19. The antisense oligomer conjugate of any one of claims 13-18, or a pharmaceutically acceptable salt thereof, wherein m is 0.
20. The antisense oligomer conjugate of any one of claims 13-18, or a pharmaceutically acceptable salt thereof, wherein m is 1.
21. The antisense oligomer conjugate of any one of claims 13-18, or a pharmaceutically acceptable salt thereof, wherein m is 2.
22. The antisense oligomer conjugate of any one of claims 13-18, or a pharmaceutically acceptable salt thereof, wherein m is 3.
23. The antisense oligomer conjugate of any one of claims 13-18, or a pharmaceutically acceptable salt thereof, wherein m is 4.
24. The antisense oligomer conjugate of any one of claims 13-18, or a pharmaceutically acceptable salt thereof, wherein m is 5.
25. The antisense oligomer conjugate of any one of claims 1-24, wherein the antisense oligomer conjugate is a free base.

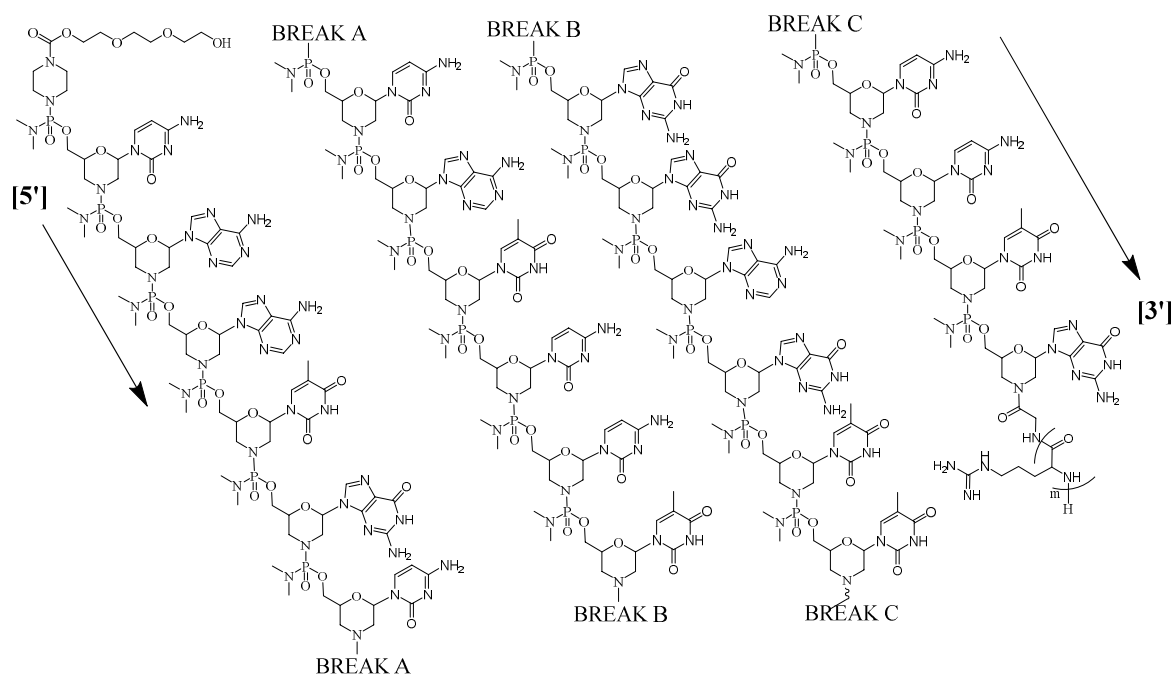
26. The antisense oligomer conjugate of any one of claims 1-24, wherein the antisense oligomer conjugate is a pharmaceutically acceptable salt.
27. The antisense oligomer conjugate of any one of claims 1-24 or 26, wherein the antisense oligomer conjugate is a hydrochloride salt.
28. A pharmaceutical composition, comprising an antisense oligonucleotide conjugate of any one of claims 1-27, or a pharmaceutically acceptable salt thereof, and a pharmaceutically acceptable carrier.
29. The pharmaceutical composition of claim 28, wherein the pharmaceutical composition is formulated for parenteral use.
30. Use of the antisense oligomer conjugate of any one of claims 1-27, or a pharmaceutically acceptable salt thereof in the manufacture of a medicament for the treatment of Duchenne muscular dystrophy (DMD) in a patient in need thereof who has a mutation that is amenable to exon skipping, wherein the medicament is to be administered to the patient.
31. The use of claim 30, wherein the antisense oligomer conjugate causes skipping of an exon in the human dystrophin gene.
32. The use of claim 30 or 31, wherein the exon is chosen from exon 44, 45, 50, 51, 52, or 53.
33. The use of any one of claims 30-32, wherein the exon is chosen from exon 45, 51, or 53.
34. Use of the antisense oligomer conjugate of any one of claims 1-27, or a pharmaceutically acceptable salt thereof in the manufacture of a medicament for the treatment of Duchenne muscular dystrophy (DMD) in a patient with an antisense oligomer conjugate, wherein the medicament is to be administered to the patient.
35. Use of the composition of claim 28 or 29, and a pharmaceutically acceptable carrier in the manufacture of a medicament for the treatment of Duchenne muscular dystrophy (DMD) in a patient in need thereof who has a mutation that is amenable to exon skipping, wherein the medicament is to be administered to the patient.

36. The use of claim 35, wherein the antisense oligomer conjugate causes skipping of an exon in the human dystrophin gene.
37. The use of claim 35 or 36, wherein the exon is chosen from exon 44, 45, 50, 51, 52, or 53.
38. The use of any one of claims 35-37, wherein the exon is chosen from exon 45, 51, or 53.
39. Use of the composition of claims 28 or 29, and a pharmaceutically acceptable carrier in the manufacture of a medicament for the treatment of Duchenne muscular dystrophy (DMD) in a patient, wherein the medicament is to be administered to the patient.
40. Use of an antisense oligomer conjugate having Formula (VI):



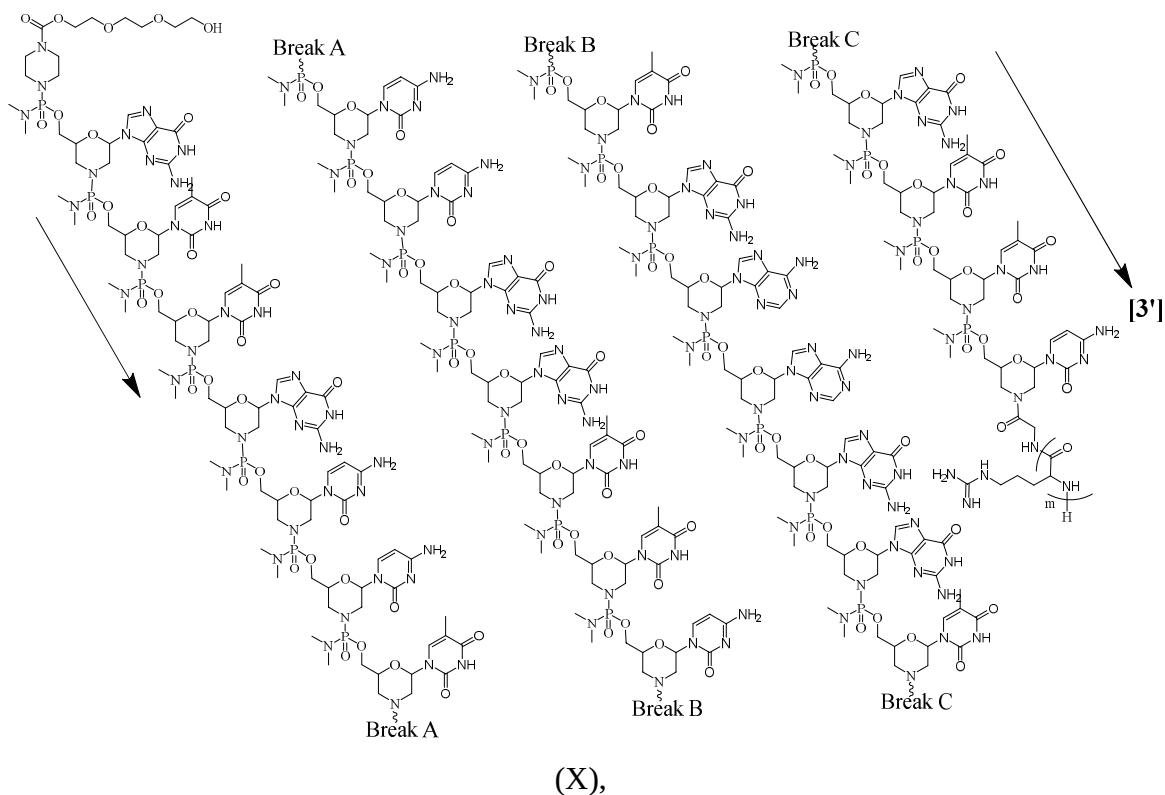
or a pharmaceutically acceptable salt thereof, wherein m is 0, 1, 2, 3, 4, or 5, in the manufacture of a medicament for the treatment of Duchenne muscular dystrophy (DMD) in a patient in need thereof who has a mutation that is amenable to exon 51 skipping, wherein the medicament is to be administered to the patient.

41. Use of an antisense oligomer conjugate having Formula (VIII):



or a pharmaceutically acceptable salt thereof, wherein m is 0, 1, 2, 3, 4, or 5, in the manufacture of a medicament for the treatment of Duchenne muscular dystrophy (DMD) in a patient in need thereof who has a mutation that is amenable to exon 45 skipping, wherein the medicament is to be administered to the patient.

42. Use of an antisense oligomer conjugate having Formula (X):



or a pharmaceutically acceptable salt thereof, wherein m is 0, 1, 2, 3, 4, or 5, in the manufacture of a medicament for the treatment of Duchenne muscular dystrophy (DMD) in a patient in need thereof who has a mutation that is amenable to exon 53 skipping, wherein the medicament is to be administered to the patient.

43. The pharmaceutical composition comprising an antisense oligonucleotide conjugate according to any one of claims 1-27, or a pharmaceutically acceptable salt thereof, for use as a medicament.
44. The antisense oligomer conjugate according to any one of claims 1-27, or a pharmaceutically acceptable salt thereof, for use as a medicament.