CLAIMS

1. A pharmaceutical composition for use in treating myotonic dystrophy type 1, wherein said pharmaceutical composition comprises a nucleic acid encoding a protein that specifically binds to the CUG repeat sequence,

said protein that specifically binds to the CUG repeat sequence comprises at least 6 pentatricopeptide repeat (PPR) motifs consisting of polypeptides of 30 - 38 amino acids long represented by Formula 1,

[Formula 1]

(Helix A)-X-(Helix B)-L (Formula 1)

[wherein in Formula 1:

Helix A is a 12 amino acids long moiety that is capable of forming an α-helix structure, and is represented by Formula 2,

[Formula 2]

A_1-A_2-A_3-A_4-A_5-A_6-A_7-A_8-A_9-A_{10}-A_{11}-A_{12} (Formula 2)

wherein in Formula 2, A_1 - A_{12} each independently represents an amino acid;

X is absent, or is a moiety consisting of a length of 1 - 9 amino acids;

Helix B is a moiety that is capable of forming an α-helix structure consisting of a length of 11 - 13 amino acids;

L is a moiety represented by Formula 3 that is 2 - 7 amino acids long;

[Formula 3]

L_{vii}-L_{vi}-L_{v}-L_{iv}-L_{iii}-L_{ii}-L_{i} (Formula 3)

and wherein in Formula 3, each amino acid is numbered from the C-terminal such as "i" (-1), "ii" (-2),

provided that L_{iii} - L_{vii} may be absent],

wherein the PPR motif that binds to C is selected from any one of (a) to (c):

(a) a PPR motif having an amino acid sequence of any one of SEQ ID NOs: 1 to 4;

(b) a PPR motif having at least 95% sequence identity to any one of the amino acid sequences of SEQ ID NOs: 1 to 4, with conservation of the amino acids at positions A1, A4, and Lii, and having binding ability to C; or

(c) a PPR motif having substitutions, additions, and/or deletions of 2 residues or less relative to any one of the amino acid sequences of SEQ ID NOs: 1 to 4, with conservation of the amino acids at positions A1, A4, and Lii, and having binding ability to C;

wherein the PPR motif that binds to U is selected from any one of (d) to (f):

(d) a PPR motif having an amino acid sequence of SEQ ID NO: 5 or 6;

(e) a PPR motif having at least 95% sequence identity to the amino acid sequence of SEQ ID NO: 5 or 6, with conservation of the amino acids at positions A1, A4, and Lii, and having binding ability to U; or

(f) a PPR motif having substitutions, additions, and/or deletions of 2 residues or less relative to the amino acid sequence of SEQ ID NO: 5 or 6, with conservation of the amino acids at positions A1, A4, and Lii, and having binding ability to U;

wherein the PPR motif that binds to G is selected from any one of (g) to (i):

(g) a PPR motif having an amino acid sequence of any one of SEQ ID NOs: 7 to 10;

(h) a PPR motif having at least 95% sequence identity to any one of the amino acid sequences of SEQ ID NOs: 7 to 10, with conservation of the amino acids at positions A1, A4, and Lii, and having binding ability to G; or

(i) a PPR motif having substitutions, additions, and/or deletions of 2 residues or less relative to any one of the amino acid sequences of SEQ ID NOs: 7 to 10, with conservation of the amino acids at positions A1, A4, and Lii, and having binding ability to G;

and thereby configured so that said protein specifically binds to said CUG repeat sequence.

2. The pharmaceutical composition for the use according to claim 1, wherein said protein that specifically binds to the CUG repeat sequence comprises 9 - 30 of said PPR motifs.

3. The pharmaceutical composition for the use according to claim 1, wherein said protein that specifically binds to the CUG repeat sequence comprises 12 - 24 of said PPR motifs.

4. The pharmaceutical composition for the use according to claim 1, characterized in that said nucleic acid encoding a protein that specifically binds to the CUG repeat sequence is integrated into an expression vector.

5. The pharmaceutical composition for the use according to claim 1, characterized in that said nucleic acid encoding a protein that specifically binds to the CUG repeat sequence is integrated into a viral vector.

6. The pharmaceutical composition for the use according to claim 1, characterized in that said viral vector is an adeno-associated virus (AAV) vector, an adenovirus vector, a retrovirus vector, a lentivirus vector, or a herpes simplex virus vector.

7. The pharmaceutical composition for the use according to claim 1, characterized in that said viral vector is an AAV vector.

8. The pharmaceutical composition for the use according to claim 1, characterized in that said AAV vector is an AAV1 vector, an AAV2 vector, an AAV6 vector, an AAV7 vector, an AAV8 vector, an AAV9 vector, an AAV10 vector, an AAV11 vector, or an AAV12 vector.

9. An expression vector comprising the nucleic acid encoding the protein that specifically binds to the CUG repeat sequence as defined in claim 1.

10. A cell comprising the expression vector according to claim 9.

11. A protein that specifically binds to the CUG repeat sequence that is produced from the cell according to claim 10.

12. The expression vector of claim 9, wherein the expression vector is a viral vector.

13. A cell comprising the viral expression vector according to claim 12.

14. Use of the pharmaceutical composition as defined in any one of claims 1 to 8 in the manufacture of a medicament for treating myotonic dystrophy type 1.