CLAIMS

1. A polynucleotide comprising the following base sequences:

(a) a base sequence encoding a fusion protein of a nuclease-deficient CRISPR effector protein and a transcriptional repressor, and

(b) a base sequence encoding a guide RNA targeting a continuous region of 18 to 24 nucleotides in length in a region set forth in SEQ ID NO: 127, SEQ ID NO: 46, SEQ ID NO: 128, SEQ ID NO: 129, SEQ ID NO: 130, SEQ ID NO: 131, SEQ ID NO: 132, SEQ ID NO: 88, SEQ ID NO: 91, SEQ ID NO: 133, SEQ ID NO: 137, SEQ ID NO: 117, or SEQ ID NO: 119 in the expression regulatory region of human DMPK gene.

2. The polynucleotide according to claim 1, comprising the following base sequences:

(a) a base sequence encoding a fusion protein of a nuclease-deficient CRISPR effector protein and a transcriptional repressor, and

(b) a base sequence encoding a guide RNA targeting a continuous region of 18 to 24 nucleotides in length in a region set forth in SEQ ID NO: 127, SEQ ID NO: 46, SEQ ID NO: 128, SEQ ID NO: 66, SEQ ID NO: 68, SEQ ID NO: 130, SEQ ID NO: 131, SEQ ID NO: 132, SEQ ID NO: 88, SEQ ID NO: 91, SEQ ID NO: 96, SEQ ID NO: 99, SEQ ID NO: 100, SEQ ID NO: 134, SEQ ID NO: 108, SEQ ID NO: 109, SEQ ID NO: 111, SEQ ID NO: 117, or SEQ ID NO: 119 in the expression regulatory region of human DMPK gene.

3. The polynucleotide according to claim 1 or 2, comprising the following base sequences:

(a) a base sequence encoding a fusion protein of a nuclease-deficient CRISPR effector protein and a transcriptional repressor, and

(b) a base sequence encoding a guide RNA targeting a continuous region of 18 to 24 nucleotides in length in a region set forth in SEQ ID NO: 63, SEQ ID NO: 136, SEQ ID NO: 83, SEQ ID NO: 99, SEQ ID NO: 135, SEQ ID NO: 109, or SEQ ID NO: 111 in the expression regulatory region of human DMPK gene.

4. The polynucleotide according to claim 1, wherein the base

sequence encoding the guide RNA comprises the base sequence set forth in SEQ ID NO: 43, SEQ ID NO: 44, SEQ ID NO: 46, SEQ ID NO: 62, SEQ ID NO: 63, SEQ ID NO: 66, SEQ ID NO: 68, SEQ ID NO: 70, SEQ ID NO: 71, SEQ ID NO: 72, SEQ ID NO: 73, SEQ ID NO: 80, SEQ ID NO: 81, SEQ ID NO: 82, SEQ ID NO: 83, SEQ ID NO: 85, SEQ ID NO: 86, SEQ ID NO: 88, SEQ ID NO: 91, SEQ ID NO: 96, SEQ ID NO: 99, SEQ ID NO: 100, SEQ ID NO: 103, SEQ ID NO: 105, SEQ ID NO: 106, SEQ ID NO: 108, SEQ ID NO: 109, SEQ ID NO: 111, SEQ ID NO: 117, or SEQ ID NO: 119, or the base sequence set forth in SEQ ID NO: 43, SEQ ID NO: 44, SEQ ID NO: 46, SEQ ID NO: 62, SEQ ID NO: 63, SEQ ID NO: 66, SEQ ID NO: 68, SEQ ID NO: 70, SEQ ID NO: 71, SEQ ID NO: 72, SEQ ID NO: 73, SEQ ID NO: 80, SEQ ID NO: 81, SEQ ID NO: 82, SEQ ID NO: 83, SEQ ID NO: 85, SEQ ID NO: 86, SEQ ID NO: 88, SEQ ID NO: 91, SEQ ID NO: 96, SEQ ID NO: 99, SEQ ID NO: 100, SEQ ID NO: 103, SEQ ID NO: 105, SEQ ID NO: 106, SEQ ID NO: 108, SEQ ID NO: 109, SEQ ID NO: 111, SEQ ID NO: 117, or SEQ ID NO: 119 in which 1 to 3 bases are deleted, substituted, inserted, and/or added.

5. The polynucleotide according to any one of claims 1 to 4, comprising at least two base sequences encoding the guide RNAs, wherein the at least two base sequences are different.

6. The polynucleotide according to any one of claims 1 to 5, wherein the transcriptional repressor is selected from the group KRAB, MeCP2, SIN3A, HDT1, MBD2B, NIPP1, and HP1A.

7. The polynucleotide according to claim 6, wherein the transcriptional repressor is KRAB.

8. The polynucleotide according to any one of claims 1 to 7, wherein the nuclease-deficient CRISPR effector protein is dCas9.

9. The polynucleotide according to claim 8, wherein the dCas9 is derived from *Staphylococcus aureus*.

10. The polynucleotide according to any one of claims 1 to 9, further comprising a promoter sequence for the base sequence encoding the guide RNA and/or a promoter sequence for the base sequence encoding the fusion protein of the nuclease-deficient CRISPR effector protein and the transcriptional repressor.

11. The polynucleotide according to claim 10, wherein the promoter sequence for the base sequence encoding the guide RNA is selected from the group U6 promoter, SNR6 promoter, SNR52 promoter, SCR1 promoter, RPR1 promoter, U3 promoter, and H1 promoter.

12. The polynucleotide according to claim 11, wherein the promoter sequence for the base sequence encoding the guide RNA is U6 promoter.

13. The polynucleotide according to any one of claims 10 to 12, wherein the promoter sequence for the base sequence encoding the fusion protein of the nuclease-deficient CRISPR effector protein and the transcriptional repressor is a ubiquitous promoter or a muscle specific promoter.

14. The polynucleotide according to claim 13, wherein the ubiquitous promoter is selected from the group EFS promoter, CMV promoter and CAG promoter.

15. The polynucleotide according to claim 13, wherein the muscle specific promoter is selected from the group CK8 promoter, myosin heavy chain kinase (MHCK) promoter, muscle creatine kinase (MCK) promoter, synthetic C5-12 (Syn) promoter, and Des promoter.

16. The polynucleotide according to claim 15, wherein the muscle specific promoter is CK8 promoter.

17. The polynucleotide according to any one of claims 10 to 16,

wherein the base sequence encoding the guide RNA comprises the base sequence set forth in SEQ ID NO: 70, SEQ ID NO: 81, SEQ ID NO: 83, or SEQ ID NO: 99, or the base sequence set forth in SEQ ID NO: 70, SEQ ID NO: 81, SEQ ID NO: 83, or SEQ ID NO: 99 in which 1 to 3 bases are deleted, substituted, inserted, and/or added,

the transcriptional repressor is KRAB,

the nuclease-deficient CRISPR effector protein is dCas9 derived from *Staphylococcus aureus*,

the promoter sequence for the base sequence encoding the

guide RNA is U6 promoter, and

the promoter sequence for the base sequence encoding the fusion protein of the nuclease-deficient CRISPR effector protein and the transcriptional repressor is CK8 promoter.

18. The polynucleotide according to claim 17,

wherein the base sequence encoding the guide RNA comprises the base sequence set forth in SEQ ID NO: 83, or the base sequence set forth in SEQ ID NO: 83 in which 1 to 3 bases are deleted, substituted, inserted, and/or added.

19. A vector comprising a polynucleotide according to any one of claims 1 to 18.

20. The vector according to claim 19, wherein the vector is a plasmid vector or a viral vector.

21. The vector according to claim 20, wherein the viral vector is selected from the group adeno-associated virus (AAV) vector, adenovirus vector, and lentivirus vector.

22. The vector according to claim 21, wherein the AAV vector is selected from the group AAV1, AAV2, AAV6, AAV7, AAV8, AAV9, Anc80, $AAV_{587}MTP$, $AAV_{588}MTP$, AAV-B1, AAVM41, and AAVrh74.

23. A pharmaceutical composition comprising a polynucleotide according to any one of claims 1 to 18 or a vector according to any one of claims 19 to 22.

24. The pharmaceutical composition according to claim 23 for treating or preventing myotonic dystrophy type 1.

25. Use of a polynucleotide according to any one of claims 1 to 18 or a vector according to any one of claims 19 to 22 for the treatment or prevention of myotonic dystrophy type 1.

26. Use of a polynucleotide according to any one of claims 1 to 18 or a vector according to any one of claims 19 to 22 in the manufacture of a pharmaceutical composition for the treatment or prophylaxis of myotonic dystrophy type 1.

27. A ribonucleoprotein comprising the following:

(c) a fusion protein of a nuclease-deficient CRISPR effector protein and a transcriptional repressor, and

(d) a guide RNA targeting a continuous region of 18 to 24 nucleotides in length in a region set forth in SEQ ID NO: 127, SEQ ID NO: 46, SEQ ID NO: 128, SEQ ID NO: 129, SEQ ID NO: 130, SEQ ID NO: 131, SEQ ID NO: 132, SEQ ID NO: 88, SEQ ID NO: 91, SEQ ID NO: 133, SEQ ID NO: 137, SEQ ID NO: 117, or SEQ ID NO: 119 in the expression regulatory region of human DMPK gene.

28. The ribonucleoprotein according to claim 27, wherein the guide RNA comprises the base sequence set forth in SEQ ID NO: 157, SEQ ID NO: 158, SEQ ID NO: 159, SEQ ID NO: 160, SEQ ID NO: 161, SEQ ID NO: 162, SEQ ID NO: 163, SEQ ID NO: 164, SEQ ID NO: 165, SEQ ID NO: 166, SEQ ID NO: 167, SEQ ID NO: 168, SEQ ID NO: 169, SEQ ID NO: 170, SEQ ID NO: 171, SEQ ID NO: 172, SEQ ID NO: 173, SEQ ID NO: 174, SEQ ID NO: 175, SEQ ID NO: 176, SEQ ID NO: 177, SEQ ID NO: 178, SEQ ID NO: 179, SEQ ID NO: 180, SEQ ID NO: 181, SEQ ID NO: 182, SEQ ID NO: 183, SEQ ID NO: 184, SEQ ID NO: 185, or SEQ ID NO: 186, or the base sequence set forth in SEQ ID NO: 157, SEQ ID NO: 158, SEQ ID NO: 159, SEQ ID NO: 160, SEQ ID NO: 161, SEQ ID NO: 162, SEQ ID NO: 163, SEQ ID NO: 164, SEQ ID NO: 165, SEQ ID NO: 166, SEQ ID NO: 167, SEQ ID NO: 168, SEQ ID NO: 169, SEQ ID NO: 170, SEQ ID NO: 171, SEQ ID NO: 172, SEQ ID NO: 173, SEQ ID NO: 174, SEQ ID NO: 175, SEQ ID NO: 176, SEQ ID NO: 177, SEQ ID NO: 178, SEQ ID NO: 179, SEQ ID NO: 180, SEQ ID NO: 181, SEQ ID NO: 182, SEQ ID NO: 183, SEQ ID NO: 184, SEQ ID NO: 185, or SEQ ID NO: 186 in which 1 to 3 bases are deleted, substituted, inserted, and/or added.

29. A composition or kit for suppressing an expression of the human DMPK gene, comprising the following:

(e) a fusion protein of a nuclease-deficient CRISPR effector protein and a transcriptional repressor, or a polynucleotide encoding the fusion protein, and

(f) a guide RNA targeting a continuous region of 18 to 24 nucleotides in length in a region set forth in SEQ ID NO: 127, SEQ ID NO: 46, SEQ ID NO: 128, SEQ ID NO: 129, SEQ ID NO: 130, SEQ ID NO: 131, SEQ ID NO: 132, SEQ ID NO: 88, SEQ ID NO: 91, SEQ ID NO:

133, SEQ ID NO: 137, SEQ ID NO: 117, or SEQ ID NO: 119 in the expression regulatory region of human DMPK gene, or a polynucleotide encoding the guide RNA.

30. The composition or kit according to claim 29, wherein the guide RNA comprises the base sequence set forth in SEQ ID NO: 157, SEQ ID NO: 158, SEQ ID NO: 159, SEQ ID NO: 160, SEQ ID NO: 161, SEQ ID NO: 162, SEQ ID NO: 163, SEQ ID NO: 164, SEQ ID NO: 165, SEQ ID NO: 166, SEQ ID NO: 167, SEQ ID NO: 168, SEQ ID NO: 169, SEQ ID NO: 170, SEQ ID NO: 171, SEQ ID NO: 172, SEQ ID NO: 173, SEQ ID NO: 174, SEQ ID NO: 175, SEQ ID NO: 176, SEQ ID NO: 177, SEQ ID NO: 178, SEQ ID NO: 179, SEQ ID NO: 180, SEQ ID NO: 181, SEQ ID NO: 182, SEQ ID NO: 183, SEQ ID NO: 184, SEQ ID NO: 185, or SEQ ID NO: 186, or the base sequence set forth in SEQ ID NO: 157, SEQ ID NO: 158, SEQ ID NO: 159, SEQ ID NO: 160, SEQ ID NO: 161, SEQ ID NO: 162, SEQ ID NO: 163, SEQ ID NO: 164, SEQ ID NO: 165, SEQ ID NO: 166, SEQ ID NO: 167, SEQ ID NO: 168, SEQ ID NO: 169, SEQ ID NO: 170, SEQ ID NO: 171, SEQ ID NO: 172, SEQ ID NO: 173, SEQ ID NO: 174, SEQ ID NO: 175, SEQ ID NO: 176, SEQ ID NO: 177, SEQ ID NO: 178, SEQ ID NO: 179, SEQ ID NO: 180, SEQ ID NO: 181, SEQ ID NO: 182, SEQ ID NO: 183, SEQ ID NO: 184, SEQ ID NO: 185, or SEQ ID NO: 186 in which 1 to 3 bases are deleted, substituted, inserted, and/or added.