【CLAIMS】

【Claim 1】

Use of a composition capable of editing a PMP22 nucleic acid sequence, comprising:

a *Streptococcus pyogenes*-derived Cas9 protein or a DNA encoding the Cas9 protein; and

a guide RNA, comprising a crRNA and a tracrRNA, or a DNA encoding the guide RNA in the manufacture of a medicament for treatment of Charcot-Marie-Tooth disease type 1A (CMT1A);

wherein the crRNA consists of 5′ –[a guide domain]-[a first complementary domain]- 3′ end;

wherein the tracrRNA consists of 5'-[second complementary donain]-[proximal domain]-[tail domain] -3' and

wherein the first complementary domain of the crRNA is bound with the second complementary domain of the tracrRNA to form a double strand,

wherein the first complementary domain and the tracrRNA are interact with the Cas9 protein to form a gRNA-Cas complex,

wherein the sequence of the guide domain comprises a corresponding RNA sequence of a target sequence of TATA-Box in the nucleic acid sequence of a PMP22 gene,

wherein the target sequence is selected from a group consisting of: SEQ ID NOs: 14 to 17.

【Claim 2】

The use of the composition according to claim 1,

wherein the guide RNA is a chimeric single stranded guide RNA,

wherein the crRNA of the guide RNA and the tracrRNA of the guide RNA are linked via a linker, and the linker is covalently linked between the crRNA and the tracrRNA.

【Claim 3】

The use of the composition according to claim 2, wherein a RNA sequence of the linker is 5′-GAAA-3′.

【Claim 4】

The use of the composition according to any one of claims 1 to 3, wherein

a sequence of the first complementary domain of the crRNA of the guide RNA consists of 5′-GUUUUAGAGCUA-3′,

wherein a sequence of the tracrRNA of the guide RNA consists of a sequence of which 5′-UAGCAAGUUAAAAU-3′, 5′-AAGGCUAGUCCG-3′, and 5′-UUAUCAACUUGAAAAAGUGGCACCGAGUCGGUGC-3′ are linked sequentially from 5′ to 3′ end.

【Claim 5】

The use of the composition according to any one of claims 1 to 4, wherein the composition composition comprises the Cas9 protein and the guide RNA,

Wherein the Cas9 protein is complexed with the guide RNA as a ribonucleoprotein(RNP).

【Claim 6】

The use of the composition according to any one of claims 1 to 4, wherein the composition comprises the DNA encoding the Cas9 protein and the DNA encoding the guide RNA.

【Claim 7】

The use of the composition according to claim 6, wherein the composition is a

viral vector comprising the DNA encoding the Cas9 protein and the DNA encoding the guide RNA.

【Claim 8】

The use of the composition of claim 7, wherein the viral vector comprises one or more selected from a retrovirus, a lentivirus, an adenovirus, adeno-associated virus (AAV), vaccinia virus, a poxvirus and a herpes simplex virus.

【Claim 9】

A composition capable of editing a PMP22 nucleic acid sequence, comprising:

a *Streptococcus pyogenes*-derived Cas9 protein or a DNA encoding the Cas9 protein; and

a guide RNA, comprising a tracrRNA, and a crRNA, or a DNA encoding the guide RNA,

wherein the crRNA consists of: 5′-[a guide domain]-[a first complementary domain]-3′,

wherein the tracrRNA consists of: 5′- second complementary domain]-[proximal domain]-[tail domain]- 3',

wherein the first complementary domain of the crRNA is bound with the second complementary domain of the tracrRNA to form a double strand,

wherein the first complementary domain and the tracrRNA can interact with the Cas9 protein to form a gRNA-Cas complex,

wherein the sequence of the guide domain comprises a corresponding RNA sequence of a target sequence of TATA Box in the nucleic acid sequence of a PMP22 gene, wherein the target sequence is selected from a group consisting of: SEQ ID NOs: 14 to 17.

【Claim 10】

The composition according to claim 9, wherein guide RNA is a chimeric single stranded guide RNA, of which the crRNA of the guide RNA and the tracrRNA of the guide RNA are covalently linked via a linker.

【Claim 11】

The composition according to claim 10, wherein the RNA sequence of the linker is 5'GAAA-3'.

【Claim 12】

The composition according to any one of claims 9 to 11, wherein the sequence of the first complementary domain of the crRNA of the guide RNA consists of 5'-GUUUUAGAGCUA-3',

wherein a sequence of the tracrRNA of the guide RNA consists of a sequence of which 5'-UAGCAAGUUAAAAU-3', 5'-AAGGCUAGUCCG-3', and 5'-UUAUCAACUUGAAAAAGUGGCACCGAGUCGGUGC-3' are linked sequentially from 5' to 3' end.

【Claim 13】

The composition according to any one of claims 9 to 12, wherein the composition is a ribonucleoprotein(RNP) comprising the Cas9 protein and the guide RNA.

【Claim 14】

The composition according to any one of claims 9 to 12, wherein the composition comprises the DNA encoding the Cas9 protein and the DNA encoding the guide RNA.

【Claim 15】

The composition according to claim 14, wherein the composition is a viral vector comprising the DNA encoding the Cas9 protein and the DNA encoding the guide RNA.

【Claim 16】

The composition according to claim 15, wherein the viral vector comprises one or

more selected from a retrovirus, a lentivirus, an adenovirus, adeno-associated virus (AAV), vaccinia virus, a poxvirus and a herpes simplex virus.

【Claim 17】

The composition according to any one of claims 9 to 16, for use in treating Charcot-Marie-Tooth disease type 1A (CMT1A).