

CLAIMS

【Claim 1】

A nucleic acid molecule comprising:
a gene expression cassette between a first inverted terminal repeat (ITR) and a second ITR,
wherein the gene expression cassette comprises a heterologous polynucleotide sequence,
wherein:
the first ITR is an adeno-associated virus (AAV) wild-type TTR, and
the second ITR consists essentially of a nucleotide sequence or a complementary sequence thereof, the nucleotide sequence of the second ITR having at least about 90% sequence identity with a nucleotide sequence of any one of SEQ ID NOs: 1 to 9,
wherein the second ITR comprises a terminal resolution site (trs) sequence and a rep-binding element (RBE) sequence,
wherein in the second ITR, all of B, B', C, C', and RBE' regions are deleted, and either A or A' region, whichever is closer to 3'-end of the (+) strand of the transgene or 5'-end of the (-) strand of the transgene, is deleted,
wherein the second ITR exists as an open-end without forming any of the stem region and the loop region of the stem-loop structure, and
wherein the second ITR retains functional activities of the trs and the RBE.

【Claim 2】

The nucleic acid molecule of claim 1, wherein the first ITR sequence and the second ITR sequence are each independently based on an ITR sequence of an AAV serotype selected from AAV1, AAV2, AAV3, AAV4, AAV5, AAV6, AAV7, AAV8, AAV9, AAV10, AAV11, and AAV12.

【Claim 3】

The nucleic acid molecule of claim 1, wherein
the first ITR is an AAV wild-type ITR, and
the second ITR at least consists essentially of a nucleotide sequence or a complementary sequence thereof, the nucleotide sequence of the second ITR having 100%

sequence identity with a nucleotide sequence of any one of SEQ ID NOs: 1 to 9, or a functional derivative thereof.

【Claim 4】

The nucleic acid molecule of claim 1, wherein
the first ITR is an AAV wild-type ITR, and
the second ITR consists essentially of any one sequence selected from nucleotide sequences of SEQ ID NOs: 1 to 9.

【Claim 5】

The nucleic acid molecule of claim 1, wherein
the first ITR is an AAV wild-type ITR, and
the second ITR consists essentially of a nucleotide sequence of SEQ ID NO: 1.

【Claim 6】

The nucleic acid molecule of claim 1, wherein the gene expression cassette further comprises at least one of a promoter, and a polyadenylation sequence.

【Claim 7】

The nucleic acid molecule of claim 1, wherein the heterologous polynucleotide sequence encodes a therapeutic gene.

【Claim 8】

A vector comprising the nucleic acid molecule of claim 1.

【Claim 9】

The vector of claim 8, wherein the vector is an AAV vector.

【Claim 10】

A composition comprising: the vector of claim 8; and a pharmaceutically acceptable carrier.

【Claim 11】

The composition of claim 10, wherein the composition is for delivering a therapeutic gene for gene therapy.