MODIFIED U1 SNRNAS

ABSTRACT

The present invention relates to modified U1 snRNAs configured to rescue exon skipping in a mutated *ABCA4* gene. In one aspect of the present invention, there is provided a modified U1 small nuclear RNA (snRNA) configured to retain exon 40 in a mature mRNA transcript of a mutated ABCA4 gene, the mutated ABCA4 gene comprising a mutation that induces skipping of exon 40, wherein the mutation is located between 3 base pairs upstream and 8 base pairs downstream of a donor splice site of exon 40, wherein the modification comprises replacing a portion of a single-stranded nucleotide sequence of a 5' region of a wild-type U1 snRNA with a single-stranded binding nucleotide sequence capable of hybridizing to a target sequence present on a pre-mRNA transcript of the mutated *ABCA4* gene, and wherein the target sequence is located in a region between 13 base pairs upstream and 15 base pairs downstream of the donor splice site of exon 40. In another aspect, there is provided a nucleic acid construct comprising a polynucleotide sequence encoding the modified U1 snRNA as described herein.

[Figure 1]