CLAIMS

1. 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.

2. The modified U1 snRNA of claim 1, wherein the mutation that induces skipping of exon 40 is c.5714+5G>A.

3. The modified U1 snRNA of claims 1 or 2, wherein the region between 13 base pairs upstream and 15 base pairs downstream of the donor splice site of exon 40 is encoded by the sequence of SEQ ID NO: 1.

4. The modified U1 snRNA of any one of claims 1 to 3, wherein the single-stranded binding nucleotide sequence comprises a sequence selected from the group consisting of SEQ ID NOs 2 to 19.

5. The modified U1 snRNA of any one of claims 1 to 4, wherein the single-stranded binding nucleotide sequence comprises a sequence selected from the group consisting of SEQ ID NOs 7, 8 and 10.

6. A nucleic acid construct comprising a polynucleotide sequence encoding the modified U1 snRNA of any one of claims 1 to 5.

7. The nucleic acid construct of claim 6, wherein the polynucleotide sequence encoding the modified U1 snRNA is operably linked to a RNU1-1 promoter and to a RNU1-1 terminator.

8. The nucleic acid construct of claims 6 or 7, comprising the sequence of SEQ ID NO: 25.

9. The nucleic acid construct of any one of claims 6 to 8, wherein a portion of the polynucleotide sequence that encodes the single-stranded binding nucleotide sequence comprises a sequence selected from the group consisting of SEQ ID NOs 26 to 43.

10. The nucleic acid construct of any one of claims 6 to 9, wherein a portion of the polynucleotide sequence that encodes the single-stranded binding nucleotide sequence comprises a sequence selected from the group consisting of SEQ ID NOs 31, 32 and 34.

11. Use of a modified U1 snRNA according to any one of claims 1 to 5 and a nucleic acid construct according to any one of claims 6 to 10 in the manufacture of a medicament for treating a *ABCA4* retinopathy.

12. The use of claim 11, wherein the *ABCA4* retinopathy is Stargardt disease.

13. A pharmaceutical composition comprising (a) a therapeutically effective amount of a modified U1 snRNA according to any one of claims 1 to 5 or a nucleic acid construct according to any one of claims 6 to 10 and (b) one or more pharmaceutically acceptable carriers and/or diluents.

14. A method of retaining exon 40 in a mature mRNA transcript of a mutated *ABCA4* gene, wherein the mutated *ABCA4* gene comprises 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, the method comprising providing a modified U1 snRNA according to any one of claims 1 to 5, wherein binding of the modified U1 snRNA to the target sequence induces the retention of exon 40 in a mature mRNA transcript of the mutated *ABCA4* gene.

15. The method of claim 14, wherein the mutation that induces skipping of exon 40 is c.5714+5G>A.

16. The method of any one of claims 14 or 15, comprising providing a modified U1 snRNA having a single-stranded binding nucleotide sequence selected from the group consisting of SEQ ID NOs 2 to 19.

17. The method of any one of claims 14 to 16, comprising providing a modified U1 snRNA having a single-stranded binding nucleotide sequence selected from the group consisting of SEQ ID NOs 7, 8 and 10.