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(71) **Applicants: AGENCY FOR SCIENCE, TECHNOLOGY AND RESEARCH** [SG/SG]; 1 Fusionopolis Way, #20-10 Connexis North Tower, Singapore 138632 (SG). **SINGAPORE HEALTH SERVICES PTE LTD** [SG/SG]; 10 Hospital Boulevard, #19-01 Outram Community Hospital, Singapore 168582 (SG).

(72) **Inventors: WEE, Keng Boon**; c/o 61 Biopolis Drive, Proteos, Singapore 138673 (SG). **SU, Xinyi**; c/o 61 Biopolis Drive, Proteos, Singapore 138673 (SG). **BANERJEE, Animesh**; c/o 61 Biopolis Drive, Proteos, Singapore 138673 (SG). **PIGINI, Paolo**; c/o 61 Biopolis Drive, Proteos, Singapore 138673 (SG). **SABAPATHY, Kanaga**; c/o 10 Hospital Boulevard, #19-01 Outram Community Hospital, Singapore 168582 (SG). **WANG, Chao**; c/o 10 Hospital

Boulevard, #19-01 Outram Community Hospital, Singapore 168582 (SG).

(74) **Agent: AMICA LAW LLC**; 77 Robinson Road, #22-01 Robinson 77, Singapore 068896 (SG).

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(54) **Title:** MODIFIED U1 SNRNAS

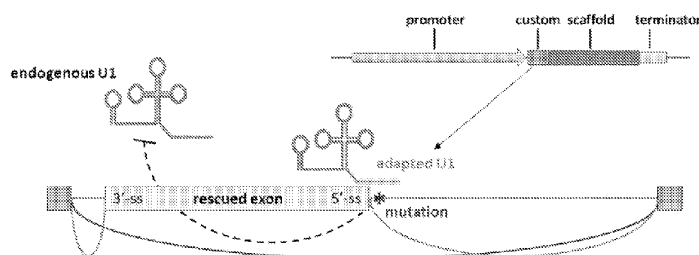


Figure 1

(57) **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.

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