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(54) Title: ALLELE-SPECIFIC KNOCKDOWN OF GENE EXPRESSION

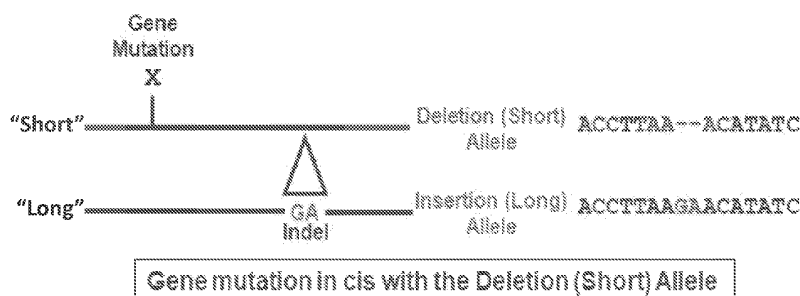


Fig. 10A

(57) Abstract: Described herein are methods of inhibiting an allele that contains a mutation in a cell (e.g., treating a subject with an allele that contains a mutation). The method includes delivering to the cell an antisense oligonucleotide that targets a variant allele that is in cis with the mutation (e.g., the mutation is a gain of function mutation or a dominant negative mutation), wherein the cell or subject is heterozygous for the target variant, and the antisense molecule is effective at knocking down expression the target variant allele (e.g., effective at preventing, treating, or ameliorating at least one symptom associated with the mutation). Also described herein are methods of haplotyping a cell or subject, and antisense oligonucleotides that target mutant superoxide dismutase 1 (SOD1) and other genes.

[Continued on next page]

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