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(54) Title: SIRNA COMPOSITIONS AND METHODS TARGETING ALPHA-SYNUCLEIN NUCLEIC ACIDS

(57) Abstract: The instant application is directed to single- or double-stranded interfering RNA molecules (e.g., siRNA) that target an alpha-synuclein (SNCA) gene. The interfering RNA molecules may contain specific patterns of nucleoside modifications and internucleoside linkage modifications, as pharmaceutical compositions including the same. The siRNA molecules may be branched siRNA molecules, such as di-branched, tri-branched, or tetra-branched siRNA molecules. The disclosed siRNA molecules may further feature a 5' phosphorus stabilizing moiety and/or a hydrophobic moiety. Additionally, the disclosure provides methods for delivering the siRNA molecule of the disclosure to the central nervous system of a subject, such as a subject identified as having a synucleinopathy.



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