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(54) Title: TARGETED CRISPR DELIVERY PLATFORMS

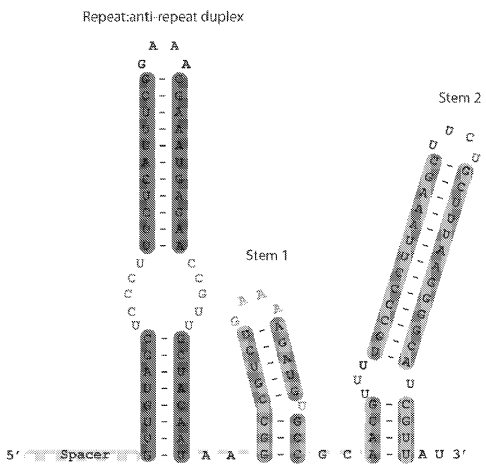


Figure 1

(57) Abstract: The present invention is related to compositions and methods for gene therapy. Several approaches described herein utilize the Neisseria meningitidis Cas9 system that provides a hyper-accurate CRISPR gene editing platform. Furthermore, the invention incorporates full length and truncated single guide RNA. sequences that permit a complete sgRNA-Nme1Cas9 vector to be inserted into an adeno-associated viral plasmid that is compatible for in vivo administration. Furthermore, Type II-C Cas9 oithologs have been identified that target protospacer adjacent motif sequences limited to between one - four required nucleotides.

[Continued on next page]

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