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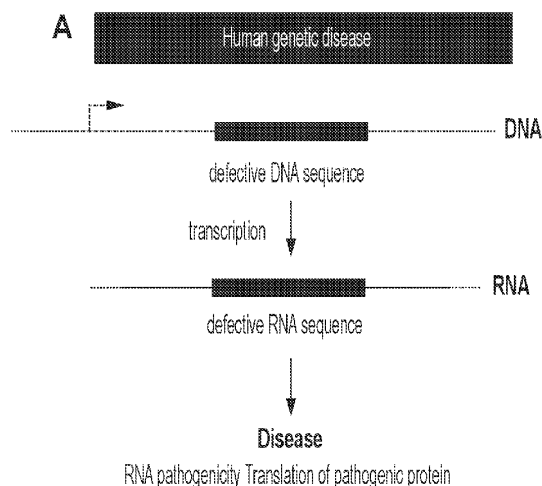
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(54) Title: HIGH EFFICIENCY TRANS-SPLICING FOR REPLACEMENT OF TARGETED RNA SEQUENCES IN HUMAN CEL-
LS

FIGURE 1



(57) Abstract: Disclosed are compositions comprising: a trans-splicing nucleic acid comprising (a) one or more replacement domains that encode a therapeutic sequence operably linked to; (b) one or more intronic domains that promote RNA splicing of the replacement domain comprising a trans-splicing enhancer sequence; and (c) one or more antisense domains that promote binding to a target RNA molecule, wherein an RNA trans-splicing reaction promotes highly efficient insertion of the replacement domain into a target RNA. Methods of making and methods of using compositions of the disclosure are also provided, including but not limited to compositions of the disclosure that may be used in the treatment of a disease or disorder in a patient or subject. Example disease or disorders of the disclosure include genetic and epigenetic diseases or disorders.

[Continued on next page]

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