[0006] In a second aspect, there is provided a DNA molecule encoding an RNA molecule as defined herein.

[0007] In a third aspect, there is provided a vector comprising an isolated nucleic acid as defined herein.

[0008] In a fourth aspect, there is provided a pharmaceutical composition comprising an isolated nucleic acid as defined herein.

[0009] In a fifth aspect, there is provided a ~~method of treating a disease comprising administering a~~ pharmaceutical composition as defined above ~~to a patient in need of~~for use in gene therapy.

Detailed Disclosure of Embodiments

[0010] Mirtrons are introns that form pre-miRNA hairpins after splicing to produce RNA interference (RNAi) effectors distinct from Drosha-dependent intronic miRNA. Disclosed herein are design principles for 3′ tailed mirtrons and corresponding mirtrons which have minimal sequence constraints as compared to canonical mirtrons. The mirtrons as defined herein may be used for delivering therapeutic RNAi and may overcome toxicity and off-target issues that are currently facing RNAi strategies.

[0011] In one example, there is provided an isolated nucleic acid molecule capable of binding a target nucleic acid sequence, wherein the isolated nucleic acid molecule comprises:

a. a stem loop structure comprising

i. a stem comprising a 5′ arm and a 3′ arm complementarily bound to each other, wherein the 5′ arm and the 3′ arm are connected to each other by a single stranded loop structure;

ii. a 5′ splice site;

18. A vector comprising an isolated nucleic acid as defined in any one of the preceding claims.

19. A pharmaceutical composition comprising an isolated nucleic acid as defined in any one of the preceding claims.

20. A ~~method of treating a disease comprising administering a~~ pharmaceutical composition of claim 19 ~~to a patient in need of~~for use in gene therapy.