Title (en)

ALTERING GENE EXPRESSION WITH ssDNA PRODUCED IN VIVO

Title (de)

ÄNSERUNG DER GENEXPRESSION DURCH IN VIVO HERGESTELLTE SSDNA

Title (fr)

MODIFICATION DE L'EXPRESSION GENETIQUE A L'AIDE D'ADN MONOCATENAIRE PRODUIT IN VIVO

Publication

EP 1222259 A1 20020717 (EN)

Application

EP 00968678 A 20001004

Priority

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- US 41156899 A 19991004
- US 51470700 A 20000228

Abstract (en)

[origin: WO0125419A1] A methods for altering expression of a target nucleic acid sequence in a target cell by production of single-stranded cDNA (ss-cDNA) in the target cell in vivo. The target cell is transfected with a cassette comprising a sequence of interest, an inverted tandem repeat, and a primer binding site 3' to the inverted tandem repeat. Transcription of the cassette by the target cell produces an RNA template which is reverse transcribed to produce ss-cDNA of a specified sequence. A reverse transcriptase/RNAse H coding gene may also be transfected into the target cell. The ss-cDNA is modified to remove all flanking vector sequences by taking advantage of the "stem-loop" structure of the ss-cDNA, which forms as a result of the inverted tandem repeat that allows the ss-cDNA to fold back on itself, forming a double stranded DNA stem. The double-stranded stem contains one or more restriction endonuclease recognition sites and the loop, which remains as ssDNA, is comprised of the sequence of interest, which can be any desired nucleotide sequence. This design allows the double-stranded stem of the stem-loop intermediate to be cleaved by the desired corresponding restriction endonuclease(s) and the loop portion, or sequence of interest, is then released as a linearized, single-stranded piece of DNA. This released (or cleaved) ssDNA piece contains minimal, if any, sequence information either upstream 5' or downstream 3' from the double stranded stem. The resulting ssDNA binds to an endogenous target nucleic acid sequence to alter the expression of that sequence for such therapeutic purposes as gene inactivation using duplex or triplex binding of nucleic acids, site-directed mutagenesis, interruption of cellular function by binding to specific cellular proteins, and interfering with RNA splicing functions.

IPC 1-7

C12N 15/10; C12N 15/11; C12N 15/63

IPC 8 full level

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