

Title (en)

\$(IN VIVO) USE OF RECOMBINAGENIC OLIGONUCLEOBASES TO CORRECT GENETIC LESIONS IN HEPATOCYTES

Title (de)

\$(IN VIVO) VERWENDUNG REKOMBINAGENISCHER OLIGONUKLEOBASEN ZUR KORREKTUR GENETISCHER DEFЕКTE IN HEPATOZYTEN

Title (fr)

UTILISATION \$(IN VIVO) D'OLIGONUCLEOBASES RECOMBINAGENES POUR CORRIGER LES LESIONS GENETIQUES DES HEPATOCYTES

Publication

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Application

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Priority

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- US 5483797 P 19970805
- US 6499697 P 19971110

Abstract (en)

[origin: WO9849350A1] The present invention concerns compositions and methods for the introduction of specific genetic changes in endogenous genes of the cells of an animal. The genetic changes are effected by oligonucleotides or oligonucleotide derivatives and analogs, which are generally less than about 100 nucleotides in length. The invention provides for macromolecular carriers, optionally incorporating ligands for clathrin coated pit receptors. In one embodiment the ligand is a lactose or galactose and the genetic changes are made in hepatocytes. By means of the invention up to 40 % of the copies of a target gene have been changed in vitro. Repair of mutant genes having a Crigler-Najjar like phenotype and Hemophilia B phenotype were observed.

IPC 1-7

C12Q 1/68; A61K 48/00; C07H 21/04

IPC 8 full level

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CPC (source: EP KR)

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