

Title (en)

NOVEL LIPID COMPLEXES FOR TRANSFERRING AT LEAST A THERAPEUTICALLY ACTIVE SUBSTANCE, IN PARTICULAR A POLYNUCLEOTIDE INTO A TARGET CELL AND USE IN GENE THERAPY

Title (de)

KOMPLEXE LIPIDE ZUR ÜBERTRAGUNG VON WENIGSTENS EINER THERAPEUTISCH WIRKSAMEN VERBINDUNG, INSBESONDERES EIN POLYNUKLEOTID, IN EINER ZIELZELLE

Title (fr)

NOUVEAUX COMPLEXES LIPIDIQUES POUR LE TRANSFERT D'AU MOINS UNE SUBSTANCE THERAPEUTIQUEMENT ACTIVE, NOTAMMENT UN POLYNUCLEOTIDE, DANS UNE CELLULE CIBLE ET UTILISATION EN THERAPIE GENIQUE

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Application

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Abstract (en)

[origin: WO9856423A1] The invention concerns a complex comprising at least a lipid and at least a therapeutically active substance useful for transferring said substance into a target cell, characterised in that said lipid is of formula (I): in which: n_1 , n_2 , identical or different are whole numbers between 0 and 1; R_1 , R_2 , identical or different are: a) selected among the group consisting of a hydrogen atom and alkyl radicals with 1 to 6 carbon atoms optionally substituted, independently of one another, by a hydroxyl radical; or b) in one particular case for which $n_1 = n_2 = 1$, R_1 and R_2 can form together a divalent alkylene chain of 2 to 3 carbon atoms (C2-C3); R_3 , R_4 , identical or different are alkyl radicals of 1 to 6 carbon atoms or can together form a divalent alkylene chain of 2 to 3 carbon atoms (C2-C3); m , p , identical or different are whole numbers between 1 and 10; R_5 , R_6 , identical or different are selected in the group consisting of radicals of formula: 1) $R_7 C(=O)-X$ in which: $X = NH$, O , S ; R_7 is an alkyl or alkenyl radical of 6 to 23 carbon atoms (C6-C23), linear or branched; 2) $R_8 R_9 NC(=O)-$ in which: R_8 , R_9 , identical or different are selected among the group consisting of the hydrogen atom or alkyl or alkenyl radicals of 6 to 23 carbon atoms, linear or branched, provided that R_8 , R_9 , cannot simultaneously correspond to the hydrogen atom; 3) and one of the radicals R_5 , R_6 can moreover correspond to the hydrogen atom. The invention also concerns the use of said complex in gene therapy.

IPC 1-7

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