

Title (en)

TARGETED DELIVERY OF GENES ENCODING ANTISENSE POLYRIBONUCLEOTIDES

Title (de)

GEZIEHLTE FREISETZUNG VON GENEN DIE FÜR GEGENLAUFIGE POLYRIBONUKLEOTIDE KODIEREN

Title (fr)

APPORT CIBLE DE GENES CODANT DES POLYRIBONUCLEOTIDES ANTISENS

Publication

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Application

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Priority

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Abstract (en)

[origin: WO9423050A1] Molecular complexes for targeting a gene encoding an antisense polyribonucleotide to a specific cell in vivo obtaining production of the polyribonucleotide within the targeted cell, and effecting specific inhibition of the expression of cellular or noncellular genes are disclosed. An expressible gene encoding a desired antisense polyribonucleotide is complexed to a conjugate of a cell-specific binding agent and a gene-binding agent. The cell-specific binding agent is specific for a cellular surface structure which mediates internalization of ligands by endocytosis. An example is the asialoglycoprotein receptor of hepatocytes. The gene-binding agent is a compound such as a polycation which stably complexes the gene under extracellular conditions and releases the gene under intracellular conditions so that it can function within a cell. The molecular complex is stable and soluble in physiological fluids and can be used in antisense gene therapy to selectively transfect cells in vivo or in vitro to provide for production of the antisense polyribonucleotide within the targeted cell, and inhibition of the expression of cellular or noncellular genes.

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