

Title (en)  
METHOD FOR VALIDATING/INVALIDATING TARGET(S) AND PATHWAYS

Title (de)  
VERFAHREN ZUR GÜLTIGKEITSERKLÄRUNG/ENTKRÄFTIGUNG VON ZIELEN UND WEGEN

Title (fr)  
PROCEDE DE VALIDATION/D'INVALIDATION DE CIBLES ET DES VOIES

Publication  
**EP 1165093 A4 20020724 (EN)**

Application  
**EP 00913730 A 20000302**

Priority  
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• US 12295099 P 19990305

Abstract (en)  
[origin: WO0051621A1] A method of determining the existence of a correlation between a function of a disease or condition and a gene or mRNA encoding a target polypeptide suspected of being associated with a disease or condition, comprises obtaining oligonucleotides (oligos) consisting of up to about 15 % adenosine (A), preferably having no adenosine content, and which is anti-sense to a target selected from the group consisting of target genes and their corresponding mRNAs, genomic and mRNA flanking regions selected from the group consisting of 3' and 5' intron-exon borders and the juxta-section between coding and non-coding regions, and all mRNA segments encoding polypeptides associated with a pre-selected disease or condition; selecting amongst the oligos one that significantly inhibits or ablates expression of the polypeptide encoded by the mRNA upon in vitro hybridization to the target mRNA; administering to a subject an amount of the selected oligo effective for in vivo hybridization to the target mRNA; and assessing a subject=s function that is associated with the disease or condition before and after administration of the oligo; wherein a change in the function=s value greater than about 70 % indicates a positive correlation, between about 40 and about 70 % a possible correlation, and below about 30 % a lack of correlation. The present method preferably administers the oligos in situ where the target is located, e.g. into the subject=s respiration when validating targets associated with malignant and other pulmonary and respiratory functions, so that the agent has direct access to the lungs. Alternatively, such desAdenosine oligos may be delivered directly to the CNS or other organs, tissues and organ systems, by means of known delivery formulations.

IPC 1-7  
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IPC 8 full level  
**C12N 15/11** (2006.01); **C12N 15/113** (2010.01); **C12Q 1/68** (2006.01); **C12Q 1/6883** (2018.01); **A61K 38/00** (2006.01)

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Citation (search report)  
• [E] WO 0042178 A2 20000720 - DU PONT PHARM CO [US]  
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• [A] POULSEN S-A ET AL: "ADENOSINE RECEPTORS: NEW OPPORTUNITIES FOR FUTURE DRUGS", BIOORGANIC & MEDICINAL CHEMISTRY, ELSEVIER SCIENCE LTD, GB, vol. 6, 1998, pages 619 - 641, XP000985735, ISSN: 0968-0896  
• See references of WO 0051621A1

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