

Title (en)
GENE THERAPY FOR CYSTIC FIBROSIS.

Title (de)
GENTHERAPIE DER MYKOVISCIDLOSES (kystic fibroses).

Title (fr)
THERAPIE GENIQUE DE LA FIBROSE KYSTIQUE.

Publication
EP 0673431 A1 19950927 (EN)

Application
EP 94903386 A 19931202

Priority
• US 9311667 W 19931202
• US 98547892 A 19921203
• US 13068293 A 19931001
• US 13674293 A 19931013

Abstract (en)
[origin: WO9412649A2] Gene Therapy vectors, which are especially useful for cystic fibrosis, and methods for using the vectors are disclosed. In preferred embodiments, the vectors are adenovirus-based. Advantages of adenovirus-based vectors for gene therapy are that they appear to be relatively safe and can be manipulated to encode the desired gene product and at the same time are inactivated in terms of their ability to replicate in a normal lytic viral life cycle. Additionally, adenovirus has a natural tropism for airway epithelia. Therefore, adenovirus-based vectors are particularly preferred for respiratory gene therapy applications such as gene therapy for cystic fibrosis. In one embodiment, the adenovirus-based gene therapy vector comprises an adenovirus 2 serotype genome in which the Ela and Elb regions of the genome, which are involved in early stages of viral replication have been deleted and replaced by genetic material of interest (e.g., DNA encoding the cystic fibrosis transmembrane regulator protein). In another embodiment, the adenovirus-based therapy vector is a pseudo-adenovirus (PAV). PAVs contain no potentially harmful viral genes, have a theoretical capacity for foreign material of nearly 36 kb, may be produced in reasonably high titers and maintain the tropism of the parent adenovirus for dividing and non-dividing human target cell types.

IPC 1-7
C12N 15/86; **C12N 15/12**; **A61K 48/00**

IPC 8 full level
C12N 15/09 (2006.01); **A61K 31/70** (2006.01); **A61K 48/00** (2006.01); **A61P 43/00** (2006.01); **C07H 21/04** (2006.01); **C07K 14/47** (2006.01); **C12N 5/10** (2006.01); **C12N 15/861** (2006.01); **A61K 38/00** (2006.01)

CPC (source: EP)
A61P 1/18 (2017.12); **A61P 11/00** (2017.12); **A61P 43/00** (2017.12); **C07K 14/4712** (2013.01); **C12N 15/86** (2013.01); **A61K 38/00** (2013.01); **A61K 48/00** (2013.01); **C12N 2710/10343** (2013.01); **C12N 2830/42** (2013.01)

Citation (search report)
See references of WO 9412649A2

Designated contracting state (EPC)
AT BE CH DE DK ES FR GB GR IE IT LI LU MC NL PT SE

DOCDB simple family (publication)
WO 9412649 A2 19940609; **WO 9412649 A3 19941110**; AU 5734994 A 19940622; AU 680459 B2 19970731; CA 2145641 A1 19940609; CA 2145641 C 20080527; CA 2592997 A1 19940609; EP 0673431 A1 19950927; EP 0905253 A2 19990331; EP 0905253 A3 20001102; EP 0911413 A2 19990428; EP 0911413 A3 20001115; EP 1024198 A2 20000802; EP 1024198 A3 20020529; JP H08503855 A 19960430

DOCDB simple family (application)
US 9311667 W 19931202; AU 5734994 A 19931202; CA 2145641 A 19931202; CA 2592997 A 19931202; EP 00201123 A 19931202; EP 94903386 A 19931202; EP 98203205 A 19931202; EP 98203206 A 19931202; JP 51348594 A 19931202