

Title (en)  
TISSUE-SPECIFIC AND PATHOGEN-SPECIFIC TOXIC AGENTS, RIBOZYMES, DNAZYMES AND ANTISENSE OLIGONUCLEOTIDES, AND METHODS OF USE THEREOF

Title (de)  
GEWEBESPEZIFISCHE UND PATHOGENSPEZIFISCHE GIFTIGE VERBINDUNGEN, RIBOZYME, DNAZYME UND ANTISENSEOLINUKLEOTIDE SOWIE ANWENDUNGEN DAFÜR

Title (fr)  
AGENTS TOXIQUES, RIBOZYMES, ANDYZMES ET OLIGONUCLEOTIDES ANTISENS SPECIFIQUES DES TISSUS ET DES PATHOGENES ET PROCEDE D'UTILISATION ASSOCIE

Publication  
**EP 1397489 A4 20050511 (EN)**

Application  
**EP 01926973 A 20010413**

Priority  
• US 0112130 W 20010413  
• US 54844900 A 20000413  
• US 25181000 P 20001207

Abstract (en)  
[origin: WO0179524A2] The present invention relates to the discovery, identification and characterization of toxic agents which are lethal to pathogens and methods for targeting such toxic agents to a pathogen or pathogen infected cells in order to treat and/or eradicate the infection. In particular, the present invention relates to toxic agents which target bacteria at different stages of the bacterial life cycle, which are delivered alone or in combination to bacteria or bacteria-infected cells. The invention relates to toxic agents which are lethal to diseased cells and methods for targeting such toxic agent to a diseased cell in order to treat and/or eradicate the disease. The present invention relates to promoter elements which are pathogen-specific or tissue-specific and the use of such promoter elements to achieve pathogen-specific or tissue-specific expression of the toxic agent(s) and/or ribozyme(s) of the present invention. Specifically, the invention relates to the delivery of one or more toxic gene products, antisense RNAs, or ribozymes, or combination thereof. The invention provides a novel system by which multiple pathogenic targets may be simultaneously targeted to cause the death of a pathogen, or cell infected with a pathogen. Further, the invention has important implications in the eradication of drug-resistant bacterium and bacterial pathogens. The invention provides a novel system by which multiple targets may be simultaneously targeted to cause the death of a diseased cell. The invention has important implications in the eradication of drug-resistant pathogens (such as antibiotic resistant bacteria) and drug-resistant diseased cells (such as drug-resistant cancer cells).

IPC 1-7  
**C12N 15/11; C12N 15/74**

IPC 8 full level  
**A61K 9/06** (2006.01); **A61K 9/127** (2006.01); **A61K 31/7088** (2006.01); **A61K 31/7105** (2006.01); **A61K 47/42** (2006.01); **A61K 48/00** (2006.01); **A61P 1/16** (2006.01); **A61P 15/00** (2006.01); **A61P 17/12** (2006.01); **A61P 31/04** (2006.01); **A61P 31/12** (2006.01); **A61P 33/00** (2006.01); **A61P 35/00** (2006.01); **C12N 9/00** (2006.01); **C12N 15/09** (2006.01); **C12N 15/11** (2006.01); **C12N 15/113** (2010.01); **C12N 15/74** (2006.01); **A61K 38/00** (2006.01)

CPC (source: EP)  
**A61K 48/00** (2013.01); **A61P 1/16** (2017.12); **A61P 15/00** (2017.12); **A61P 17/12** (2017.12); **A61P 31/04** (2017.12); **A61P 31/12** (2017.12); **A61P 33/00** (2017.12); **A61P 35/00** (2017.12); **C12N 15/113** (2013.01); **C12N 15/1131** (2013.01); **C12N 15/74** (2013.01); **A61K 38/00** (2013.01); **C12N 2310/111** (2013.01); **C12N 2310/12** (2013.01); **C12N 2310/317** (2013.01)

Citation (search report)

- [X] WO 9323569 A1 19931125 - RIBOZYME PHARM INC [US]
- [A] WO 0009673 A1 20000224 - JOHNSON & JOHNSON RES PTY LTD [AU]
- [A] WO 9703211 A1 19970130 - ISIS PHARMACEUTICALS INC [US], et al
- [E] WO 0246449 A2 20020613 - UNIV PENNSYLVANIA [US], et al
- [X] WO 9108312 A1 19910613 - GENE TRAK SYSTEMS [US]
- [X] WO 9426934 A2 19941124 - BAXTER DIAGNOSTICS INC [US], et al
- [X] DIAZ-FLORES ESTEVEZ F ET AL: "Detection of human Papillomavirus types 6b, 11, 16 and 18 in cervical scrapes by a multiplex polymerase chain reaction", REVISTA DE LA SOCIEDAD ESPANOLA DE QUIMICA CLINICA 1996 SPAIN, vol. 15, no. 6, 1996, pages 420 - 424, XP001203827, ISSN: 0213-8514
- [PA] PAN WEI-HUA ET AL: "A selection system for identifying accessible sites in target RNAs", RNA (NEW YORK), vol. 7, no. 4, April 2001 (2001-04-01), pages 610 - 621, XP002317752, ISSN: 1355-8382
- [A] WELCH P J ET AL: "Intracellular application of hairpin ribozyme genes against hepatitis B virus.", GENE THERAPY. JUL 1997, vol. 4, no. 7, July 1997 (1997-07-01), pages 736 - 743, XP002317753, ISSN: 0969-7128
- [A] WEIZSAECKER VON F ET AL: "CLEAVAGE OF HEPATITIS B VIRUS RNA BY THREE RIBOZYMES TRANSCRIBED FROM A SINGLE DNA TEMPLATE", BIOCHEMICAL AND BIOPHYSICAL RESEARCH COMMUNICATIONS, ACADEMIC PRESS INC. ORLANDO, FL, US, vol. 189, no. 2, 15 December 1992 (1992-12-15), pages 743 - 748, XP000652045, ISSN: 0006-291X
- [X] CHEN Z ET AL: "EFFECTIVENESS OF THREE RIBOZYMES FOR CLEAVAGE OF AN RNA TRANSCRIPT FROM HUMAN PAPILLOMAVIRUS TYPE 18", CANCER GENE THERAPY, NORWALK, CT, US, vol. 2, no. 4, 1995, pages 263 - 271, XP008040034, ISSN: 0929-1903
- [X] STEELE C ET AL: "EFFECTS OF HUMAN PAPILLOMAVIRUS TYPE 18-SPECIFIC ANTISENSE OLIGONUCLEOTIDES ON THE TRANSFORMED PHENOTYPE OF HUMAN CARCINOMA CELL LINES", CANCER RESEARCH, AMERICAN ASSOCIATION FOR CANCER RESEARCH, BALTIMORE, MD, US, vol. 53, no. 10, 15 May 1993 (1993-05-15), pages 2330 - 2337, XP002030937, ISSN: 0008-5472
- [A] PASSMAN M ET AL: "In situ demonstration of inhibitory effects of hammerhead ribozymes that are targeted to the hepatitis Bx sequence in cultured cells.", BIOCHEMICAL AND BIOPHYSICAL RESEARCH COMMUNICATIONS. 24 FEB 2000, vol. 268, no. 3, 24 February 2000 (2000-02-24), pages 728 - 733, XP002317754, ISSN: 0006-291X
- [A] JI W ET AL: "Inhibition of hepatitis B virus by retroviral vectors expressing antisense RNA.", JOURNAL OF VIRAL HEPATITIS. MAY 1997, vol. 4, no. 3, May 1997 (1997-05-01), pages 167 - 173, XP002317755, ISSN: 1352-0504
- [A] ALVAREZ-SALAS L M ET AL: "Inhibition of HPV-16 E6/E7 immortalization of normal keratinocytes by hairpin ribozymes.", PROCEEDINGS OF THE NATIONAL ACADEMY OF SCIENCES OF THE UNITED STATES OF AMERICA. 3 FEB 1998, vol. 95, no. 3, 3 February 1998 (1998-02-03), pages 1189 - 1194, XP002317756, ISSN: 0027-8424
- [A] PLOURDE ROBERT ET AL: "Targeted therapy for viral hepatitis", ADVANCED DRUG DELIVERY REVIEWS, vol. 17, no. 3, 1995, pages 311 - 315, XP002317757, ISSN: 0169-409X

- [A] VENTURINI FRANCESCA ET AL: "Kinetic selection of HPV 16 E6/E7-directed antisense nucleic acids: Anti-proliferative effects on HPV 16-transformed cells", NUCLEIC ACIDS RESEARCH, OXFORD UNIVERSITY PRESS, SURREY, GB, vol. 27, no. 7, 1 April 1999 (1999-04-01), pages 1585 - 1592, XP002234550, ISSN: 0305-1048
- See references of WO 0179524A2

Designated contracting state (EPC)

AT BE CH CY DE DK ES FI FR GB GR IE IT LI LU MC NL PT SE TR

DOCDB simple family (publication)

**WO 0179524 A2 20011025; WO 0179524 A3 20040108**; AU 2001253471 B2 20070726; AU 5347101 A 20011030; CA 2406403 A1 20011025; EP 1397489 A2 20040317; EP 1397489 A4 20050511; JP 2004525602 A 20040826

DOCDB simple family (application)

**US 0112130 W 20010413**; AU 2001253471 A 20010413; AU 5347101 A 20010413; CA 2406403 A 20010413; EP 01926973 A 20010413; JP 2001577507 A 20010413