

(12) INTERNATIONAL APPLICATION PUBLISHED UNDER THE PATENT COOPERATION TREATY (PCT)

(19) World Intellectual Property Organization
International Bureau



(43) International Publication Date
28 March 2002 (28.03.2002)

PCT

(10) International Publication Number
WO 02/024234 A3

(51) International Patent Classification⁷: C12N 15/12,
C07K 14/50, 14/52, 14/71, A61K 48/00, A01K 67/027

(21) International Application Number: PCT/US01/29480

(22) International Filing Date:
20 September 2001 (20.09.2001)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
09/665,493 20 September 2000 (20.09.2000) US

(71) Applicant (for all designated States except US): THE
REGENTS OF THE UNIVERSITY OF CALIFORNIA
[US/US]; 1111 Franklin Street, 12th floor, Oakland, CA
94607-5200 (US).

(72) Inventors; and

(75) Inventors/Applicants (for US only): MANNING,
William, C., Jr. [US/US]; 3660 Country Club Drive,
Redwood City, CA 94061 (US). DWARKI, Varavani,
J. [US/US]; 16 Grove Farm Road, Pittstown, NJ 08867
(US). RENDAHL, Katherine [US/US]; 1221 Grizzly
Peak Boulevard, Berkeley, CA 94708 (US). ZHOU,
Shangzhen [CN/US]; 125 Fundy Bay, Alameda, CA

94502 (US). McGEE, Laura, H. [US/US]; 652 54th
Street, Oakland, CA 94609 (US). LAU, Dana [US/US];
Apartement A, 280 Lenox Avenue, Oakland, CA 94610
(US). FLANNERY, John, G. [US/US]; 1728 Marin Av-
enue, Berkeley, CA 94707 (US). MILLER, Sheldon, S.
[US/US]; 186 Hillcrest Road, Berkeley, CA 94705 (US).
WANG, Fei [CN/US]; 981 9th Street, Albany, CA 94710
(US). DIPOLO, Adriana [VE/CA]; 704 Champagneur
Avenue, Outremont, Quebec H2V 3P8 (CA).

(74) Agents: CULLMAN, Louis, C. et al.; Oppenheimer Wolff
& Donnelly LLP, 840 Newport Center Drive, Suite 700,
Newport Beach, CA 92660-7007 (US).

(81) Designated States (national): AU, CA, JP, US.

(84) Designated States (regional): European patent (AT, BE,
CH, CY, DE, DK, ES, FI, FR, GB, GR, IE, IT, LU, MC,
NL, PT, SE, TR).

Published:

— with international search report

(88) Date of publication of the international search report:
27 December 2002

For two-letter codes and other abbreviations, refer to the "Guid-
ance Notes on Codes and Abbreviations" appearing at the begin-
ning of each regular issue of the PCT Gazette.



WO 02/024234 A3

(54) Title: USE OF RECOMBINANT GENE DELIVERY VECTORS FOR TREATING OR PREVENTING DISEASES OF THE EYE

(57) Abstract: Gene delivery vectors, such as, for example, recombinant adeno-associated viral vectors, and methods of using such vectors are provided for use in treating or preventing diseases of the eye.

INTERNATIONAL SEARCH REPORT

Interr. Application No
PC1/US 01/29480

A. CLASSIFICATION OF SUBJECT MATTER
 IPC 7 C12N15/12 C07K14/50 C07K14/52 C07K14/71 A61K48/00
 A01K67/027

According to International Patent Classification (IPC) or to both national classification and IPC

B. FIELDS SEARCHED
 Minimum documentation searched (classification system followed by classification symbols)
 IPC 7 C07K A01K C12N A61K

Documentation searched other than minimum documentation to the extent that such documents are included in the fields searched

Electronic data base consulted during the international search (name of data base and, where practical, search terms used)
 EPO-Internal, WPI Data, MEDLINE, BIOSIS

C. DOCUMENTS CONSIDERED TO BE RELEVANT

Category °	Citation of document, with indication, where appropriate, of the relevant passages	Relevant to claim No.
X	WO 00 15822 A (JANSSEN JACQUES J ;CATHOLIC UNIVERSITY NIJMEGEN (NL); HAUSWIRTH WI) 23 March 2000 (2000-03-23)	1-16, 21-25,29
Y	the whole document ---	5-19
X	WO 99 66959 A (LOGAN ANN ;BERRY MARTIN (GB); BAIRD ANDREW (US); GONZALEZ ANA MARI) 29 December 1999 (1999-12-29)	1-4,15, 16,22, 23,29
Y	the whole document ---	5-14,27, 28
	-/--	

Further documents are listed in the continuation of box C. Patent family members are listed in annex.

° Special categories of cited documents :

"A" document defining the general state of the art which is not considered to be of particular relevance	"T" later document published after the international filing date or priority date and not in conflict with the application but cited to understand the principle or theory underlying the invention
"E" earlier document but published on or after the international filing date	"X" document of particular relevance; the claimed invention cannot be considered novel or cannot be considered to involve an inventive step when the document is taken alone
"L" document which may throw doubts on priority claim(s) or which is cited to establish the publication date of another citation or other special reason (as specified)	"Y" document of particular relevance; the claimed invention cannot be considered to involve an inventive step when the document is combined with one or more other such documents, such combination being obvious to a person skilled in the art.
"O" document referring to an oral disclosure, use, exhibition or other means	"&" document member of the same patent family
"P" document published prior to the international filing date but later than the priority date claimed	

Date of the actual completion of the international search 21 May 2002	Date of mailing of the international search report 16. 08. 2002
---	---

Name and mailing address of the ISA European Patent Office, P.B. 5818 Patentlaan 2 NL - 2280 HV Rijswijk Tel. (+31-70) 340-2040, Tx. 31 651 epo nl, Fax: (+31-70) 340-3016	Authorized officer Schulz, R
--	--

INTERNATIONAL SEARCH REPORT

International Application No

PC1/US 01/29480

C.(Continuation) DOCUMENTS CONSIDERED TO BE RELEVANT		
Category	Citation of document, with indication, where appropriate, of the relevant passages	Relevant to claim No.
X	AKIMOTO M ET AL: "ADENOVIRALLY EXPRESSED BASIC FIBROBLAST GROWTH FACTOR RESCUES PHOTORECEPTOR CELLS IN RCS RATS" INVESTIGATIVE OPHTHALMOLOGY & VISUAL SCIENCE, ASSOCIATION FOR RESEARCH IN VISION AND, US, vol. 40, no. 2, 1999, pages 273-279, XP000917329 ISSN: 0146-0404 the whole document	22-24,29
X	--- CAYOUILLE M ET AL: "ADENOVIRUS-MEDIATED GENE TRANSFER OF CILIARY NEUROTROPHIC FACTOR CAN PREVENT PHOTORECEPTOR DEGENERATION IN THE RETINAL DEGENERATION (RD) MOUSE" HUMAN GENE THERAPY, vol. 8, no. 4, 1 March 1997 (1997-03-01), pages 423-430, XP000876513 ISSN: 1043-0342 the whole document	22,23
X	--- WO 98 13071 A (KENDALL RICHARD L ;THOMAS KENNETH A JR (US); BETT ANDREW J (US); G) 2 April 1998 (1998-04-02)	22,26,29
Y	page 5 -page 10	17-20, 27,28
Y	--- WO 99 36511 A (DUBENSKY THOMAS W ;SHERIDAN PHILIP LEE (US); CHIRON CORP (US); HSU) 22 July 1999 (1999-07-22) the whole document	15,16, 20,27,28
P,X, L	--- WO 00 54813 A (DWARKI VARAVANI J ;RENDAHL KATHERINE (US); CHIRON CORP (US); MCGEE) 21 September 2000 (2000-09-21) L: priority the whole document	1-29
P,X	--- WO 00 71582 A (HUMAN GENOME SCIENCES INC ;GREENE JOHN M (US); MELDER ROBERT (US);) 30 November 2000 (2000-11-30) the whole document -----	1-3,5,6, 9,16,17, 19, 21-24,29

INTERNATIONAL SEARCH REPORT

International application No.
PCT/US 01/29480

Box I Observations where certain claims were found unsearchable (Continuation of item 1 of first sheet)

This International Search Report has not been established in respect of certain claims under Article 17(2)(a) for the following reasons:

1. Claims Nos.: -
because they relate to subject matter not required to be searched by this Authority, namely:
see FURTHER INFORMATION sheet PCT/ISA/210

2. Claims Nos.:
because they relate to parts of the International Application that do not comply with the prescribed requirements to such an extent that no meaningful International Search can be carried out, specifically:

3. Claims Nos.:
because they are dependent claims and are not drafted in accordance with the second and third sentences of Rule 6.4(a).

Box II Observations where unity of invention is lacking (Continuation of item 2 of first sheet)

This International Searching Authority found multiple inventions in this international application, as follows:

see additional sheet

1. As all required additional search fees were timely paid by the applicant, this International Search Report covers all searchable claims.

2. As all searchable claims could be searched without effort justifying an additional fee, this Authority did not invite payment of any additional fee.

3. As only some of the required additional search fees were timely paid by the applicant, this International Search Report covers only those claims for which fees were paid, specifically claims Nos.:

4. No required additional search fees were timely paid by the applicant. Consequently, this International Search Report is restricted to the invention first mentioned in the claims; it is covered by claims Nos.:

1-16,23-25 complete 22, 27-29 partial

Remark on Protest

- The additional search fees were accompanied by the applicant's protest.
- No protest accompanied the payment of additional search fees.

FURTHER INFORMATION CONTINUED FROM PCT/ISA/ 210

This International Searching Authority found multiple (groups of) inventions in this international application, as follows:

1. Claims: 1 - 16, 23 - 25 (complete); 22, 27 - 29 (partial)

Method of treating and preventing eye diseases comprising intraocular administration of a gene delivery vector which directs expression of a neurotrophic factor in the eye; and said gene delivery vector.

2. Claims: 17 - 21, 26 (complete); 22, 27 - 29 (partial)

Method of inhibiting neovascular disease of the eye comprising intraocular administration of a gene delivery vector which directs the expression of an anti-angiogenic factor to inhibit the neovascular disease of the eye; and said gene delivery vector.

3. Claims: 30 - 44 (complete)

A non-human animal model of neovascularisation of the eye comprising an animal having an angiogenic transgene in the eye;
a method of generating said non-human animal model; and
methods using said non human-animal model.

FURTHER INFORMATION CONTINUED FROM PCT/ISA/ 210

Continuation of Box I.1

Although claims 1 - 21 are directed to a method of treatment of the human/animal body, the search has been carried out and based on the alleged effects of the compound/composition.

Continuation of Box I.1

Rule 39.1(iv) PCT - Method for treatment of the human or animal body by therapy

INTERNATIONAL SEARCH REPORT

Information on patent family members

International Application No

PCT/US 01/29480

Patent document cited in search report		Publication date	Patent family member(s)	Publication date
WO 0015822	A	23-03-2000	AU 6394199 A	03-04-2000
			EP 1114171 A1	11-07-2001
			WO 0015822 A1	23-03-2000

WO 9966959	A	29-12-1999	AU 6238099 A	10-01-2000
			CA 2330026 A1	29-12-1999
			EP 1082142 A2	14-03-2001
			JP 2002518464 T	25-06-2002
			WO 9966959 A2	29-12-1999

WO 9813071	A	02-04-1998	AU 738806 B2	27-09-2001
			AU 4650397 A	17-04-1998
			EP 0928203 A1	14-07-1999
			JP 2001501471 T	06-02-2001
			WO 9813071 A1	02-04-1998
			US 6375929 B1	23-04-2002

WO 9936511	A	22-07-1999	US 2002048805 A1	25-04-2002
			AU 2329599 A	02-08-1999
			CA 2318575 A1	22-07-1999
			EP 1045921 A2	25-10-2000
			WO 9936511 A2	22-07-1999
			US 2002068354 A1	06-06-2002

WO 0054813	A	21-09-2000	AU 3755900 A	04-10-2000
			EP 1183051 A2	06-03-2002
			WO 0054813 A2	21-09-2000

WO 0071582	A	30-11-2000	AU 5019700 A	12-12-2000
			WO 0071582 A1	30-11-2000
