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



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(54) Title: VECTORS AND TRANSGENIES WITH REGULATORY ELEMENTS FOR GENE DELIVERY TO THE LIVER

(57) Abstract: The invention is directed to novel combinations of liver specific enhancers and promoter elements for achieving persistent transgene expression in the liver. The liver specific enhancer elements may be derived from either the human serum albumin, prothrombin, α -1microglobulin or aldolase genes in single copies or in multimerized form linked to elements derived from the cytomegalovirus intermediate early (CMV), α -1-antitrypsin or albumin promoters. In a preferred embodiment of the invention, an adenoviral vector comprising a liver specific enhancer/promoter combination operably linked to a transgene is administered to recipient cells. In other embodiments of the invention, adeno-associated viral vectors, retroviral vectors, lentiviral vectors or a plasmid comprising the liver specific enhancer/promoter combination linked to a transgene is administered to recipient cells. Also within the scope of the invention are promoter elements derived from the human prothrombin gene and the β -fibrinogen gene.

INTERNATIONAL SEARCH REPORT

National Application No
PCT/US 00/31444

A. CLASSIFICATION OF SUBJECT MATTER

IPC 7 C12N15/85 C12N15/86

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)

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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, 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 99 29848 A (IMMUNE RESPONSE CORP INC) 17 June 1999 (1999-06-17)	1-3
A	abstract examples claims	4-14
X	WO 99 36557 A (MARSHALL JOHN ; ARMENTANO DONNA (US); GENZYME CORP (US); YEW NELSON) 22 July 1999 (1999-07-22) the whole document especially examples 1, 2 and claims.	1-3, 8-10
X	WO 97 04117 A (MAX PLANCK GESELLSCHAFT ; SANDIG VOLKER (DE); LOESER PETER (DE); ST) 6 February 1997 (1997-02-06) the whole document	1-3, 8-10
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☒ Further documents are listed in the continuation of box C.

☒ Patent family members are listed in annex.

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- *8* document member of the same patent family

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C.(Continuation) DOCUMENTS CONSIDERED TO BE RELEVANT

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A	page 7 -page 10 examples claims	8-10
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Information on patent family members

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