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(54) Title: DOUBLE-STRANDED/SELF-COMPLEMENTARY VECTORS WITH A TRUNCATED CBA PROMOTER AND METHODS OF GENE DELIVERY

(57) Abstract: The present invention concerns vectors carrying a truncated chimeric CMV- chicken  $\beta$ -actin (smCBA) promoter in which the hybrid chicken  $\beta$ -actin/rabbit  $\beta$ -globin intron is greatly shortened, and their use to deliver to an operatively linked polynucleotide to host cells in vitro or in vivo, resulting in expression of the polynucleotide in the host cells. In one embodiment, the vector carrying the smCBA promoter is administered to the eye. In another embodiment, the vector carrying the smCBA promoter is a self-complementary adeno-associated virus (AAV). The AAV vector may be of any serotype (e.g., type 1, type 2, type 3, type 4, type 5, type 6, type 7, type 8, type 9, type 10). In another embodiment, a self-complementary vector carrying the smCBA promoter is administered to the eye. Another aspect of the invention concerns host cells carrying a vector of the invention. Another aspect of the invention concerns pharmaceutical composition comprising the vectors or host cells of the invention, and a pharmaceutically acceptable carrier.



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# INTERNATIONAL SEARCH REPORT

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## A. CLASSIFICATION OF SUBJECT MATTER

IPC: C12N 15/00( 2006.01), 5/00( 2006.01); A61K 38/00( 2006.01)

USPC: 435/320.1, 325, 455; 514/44

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)

U.S. : 435/320.1, 325, 455; 514/44

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 practicable, search terms used)  
east, biosis caplus, medline

## C. DOCUMENTS CONSIDERED TO BE RELEVANT

| Category * | Citation of document, with indication, where appropriate, of the relevant passages                                                                                     | Relevant to claim No.              |
|------------|------------------------------------------------------------------------------------------------------------------------------------------------------------------------|------------------------------------|
| X          | GONG et al. Recombinant adeno-associated virus serotype 2 effectively transduces primary rat brain astrocytes and microglia. Brain Research, Vol. 14, No. 1, pg 18-24. | 1, 3, 4, 6, 7-15, 17-20, 25-27, 29 |

☐ Further documents are listed in the continuation of Box C.

☐ See patent family annex.

\* Special categories of cited documents:

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