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(54) Title: METHODS AND COMPOSITIONS FOR INCREASING SIALIC ACID PRODUCTION AND TREATING SIALIC RELATED DISEASE CONDITIONS

(57) Abstract: Disclosed herein are methods of expressing UDP-GlcNAc 2-Epimerase/ManNAc Kinase enzyme (GNE) peptide in a cell of a subject comprising: delivering into the cell of the subject an isolated nucleic acid expression construct that comprises a promoter operatively linked to a nucleic acid sequence encoding a GNE peptide or a therapeutically active fragment thereof, wherein the GNE peptide has the amino acid sequence of SEQ ID NO:3, wherein upon the delivering into the cell of the subject, the nucleic acid expression construct initiates expression of the GNE peptide or a therapeutically active fragment thereof. Also disclosed are methods of producing a GNE peptide in a cell comprising infecting the cell with an isolated nucleic acid construct that comprises a promoter operatively linked to a nucleic acid sequence encoding a GNE peptide or a therapeutically active fragment thereof, wherein the GNE peptide has the amino acid sequence of SEQ ID NO:3.



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INTERNATIONAL SEARCH REPORT

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International application No.

PCT/US 12/23536

A. CLASSIFICATION OF SUBJECT MATTER

IPC(8) - A61K 48/00, C12N 15/00, C12N 15/07, C12N 5/10, A01K 67/00 (2012.01)

USPC - 514/44R

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 -- A61K 48/00, C12N 15/00, C12N 15/07, C12N 5/10, A01K 67/00; USPC -- 514/44R

Documentation searched other than minimum documentation to the extent that such documents are included in the fields searched :
USPC- 435/320.1, 435/352, 800/13 (text search)

Electronic data base consulted during the international search (name of data base and, where practicable, search terms used)

PubWEST -- PGPB,USPT,USOC,EPAB,JPAB; Dialog Classic Files -- 654, 652, 349, 348, 340, 35, 65, 155; USPTO Web Page; PCT Patentscope; Google Scholar; Search terms -- GNE expression, vector construct, transfection, mammalian cell, promoter, limb injection, pressure, extravasation, intramuscular perfusion, single dose, polynucleotide sequence

C. DOCUMENTS CONSIDERED TO BE RELEVANT

Category*	Citation of document, with indication, where appropriate, of the relevant passages	Relevant to claim No.
X -- Y -- A	WO 2008/097623 A2 (MAPLES et al.) 14 August 2008 (14.08.2008) para [0004]-[0006], [0020], [0031]-[0036], [0040], [0042], [0048], [0055], [0071], SEQ ID NO: 11, Fig 1, abstract	1, 11-13, 17, 18 ----- 3-7, 15, 16 ----- 2, 8-10, 14, 19
Y	US 2009/0298112 A1 (DARVISH et al.) 03 December 2009 (03.12.2009) para [0006]-[0010], [0057], [0078], SEQ ID NO: 3	3-7
Y	US 2007/0073264 A1 (STEDMAN et al.) 29 March 2007 (29.03.2007) para [0017], [0028], [0057]-[0060], [0069], [0078], [0123], [0124], [0126], [0129]	4-7, 15, 16

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

* Special categories of cited documents:

"A" document defining the general state of the art which is not considered to be of particular relevance

"E" earlier application or patent but published on or after the international filing date

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"O" document referring to an oral disclosure, use, exhibition or other means

"P" document published prior to the international filing date but later than the priority date claimed

"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

"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

"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

"&" document member of the same patent family

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Box No. I Nucleotide and/or amino acid sequence(s) (Continuation of item 1.c of the first sheet)

1. With regard to any nucleotide and/or amino acid sequence disclosed in the international application, the international search was carried out on the basis of a sequence listing filed or furnished:

a. (means)

☐

on paper

☒

in electronic form

b. (time)

☐

in the international application as filed

☒

together with the international application in electronic form

☐

subsequently to this Authority for the purposes of search

2. ☐ In addition, in the case that more than one version or copy of a sequence listing has been filed or furnished, the required statements that the information in the subsequent or additional copies is identical to that in the application as filed or does not go beyond the application as filed, as appropriate, were furnished.

3. Additional comments: