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13 October 2011

(54) Title: ASSISTED ENZYME REPLACEMENT THERAPY

(57) Abstract: Reagents and methods useful for the synthesis of conjugates comprising guanidinylated cyclic acetals are provided. Also provided are methods for increasing the cellular uptake of various therapeutic compounds and treatment modalities using these conjugates.



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A. CLASSIFICATION OF SUBJECT MATTER

A61K 31/7036(2006.01)i, A61K 31/7048(2006.01)i, A61K 38/43(2006.01)i, A61K 31/70(2006.01)i, A61K 31/352(2006.01)i, A61P 43/00(2006.01)i, A61P 3/00(2006.01)i

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)

A61K 31/7036; A61K 38/47

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

Korean utility models and applications for utility models
Japanese utility models and applications for utility models

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

eKOMPASS(KIPO internal) & Keywords: Lysosomal storage disease, guanidinoglycoside, guanidinyle, aminoglycoside

C. DOCUMENTS CONSIDERED TO BE RELEVANT

Category*	Citation of document, with indication, where appropriate, of the relevant passages	Relevant to claim No.
Y	US 6537785 B1 (CANFIELD,W.M.) 25 March 2003 See claims; column 3, line 12-15; examples 26-28.	1-27,31-40,42
Y	ELSON-SCHWAB,L. et al., "Guanidinylated Neomycin Delivers Large, Bioactive Cargo into Cells through a Heparan sulfate-dependent Pathway", Journal of Biological Chemistry, 2007, Vol.282, No.18, pages 13585-13591 See abstract; the whole document; page 13589, right column, line 40-42.	1-27,31-40,42
Y	LUEDTKE,N.W. et al., "Cellular Uptake of Aminoglycosides, Guanidinoglycosides, and Poly-arginine" J. Am. Chem. Soc., 2003, Vol.125, pages 12374-12375 See abstract; the whole document.	1-27,31-40,42
A	DESNICK,R.J. "Enzyme Replacement and Enhancement Therapies for Lysosomal Disease" J. Inherit. Metab. Dis., 2004, Vol.27, pages 385-410 See the whole document.	1-27,31-40,42
A	WINCHESTER,B. et al., "The Molecular Basis of Lysosomal Storage Diseases and Their Treatment" Biochemical Society Transactions, 2000, Vol.28, part 2, pages 150-153 See the whole document.	1-27,31-40,42

Further documents are listed in the continuation of Box C.

See patent family annex.

* 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

"L" document which may throw doubts on priority claim(s) or which is cited to establish the publication date of citation or other special reason (as specified)

"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

Date of the actual completion of the international search

27 JUNE 2011 (27.06.2011)

Date of mailing of the international search report

28 JUNE 2011 (28.06.2011)

Name and mailing address of the ISA/KR



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Box No. II Observations where certain claims were found unsearchable (Continuation of item 2 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.: 28-30,41,43-45
because they relate to subject matter not required to be searched by this Authority, namely:
Claims 28-30,41,43-45 pertain to methods for the treatment of the human or animal body by therapy, and thus relate to a subject matter which this International Searching Authority is not required, under Article 17(2)(a)(i) of the PCT and Rule 39.1(iv) of the Regulations under PCT, to search.
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 No. III Observations where unity of invention is lacking (Continuation of item 3 of first sheet)

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

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.:

Remark on Protest

- The additional search fees were accompanied by the applicant's protest and, where applicable, the payment of a protest fee.
- The additional search fees were accompanied by the applicant's protest but the applicable protest fee was not paid within the time limit specified in the invitation.
- No protest accompanied the payment of additional search fees.

INTERNATIONAL SEARCH REPORT

Information on patent family members

International application No.

PCT/US2010/048968

Patent document cited in search report	Publication date	Patent family member(s)	Publication date
US 6537785 B1	25.03.2003	None	