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Published:

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- with sequence listing part of description (Rule 5.2(a))

(88) Date of publication of the international search report:

27 December 2012

(54) Title: RECOMBINANT VIRUS PRODUCTS AND METHODS FOR INHIBITION OF EXPRESSION OF MYOTILIN

(57) Abstract: The present invention relates to RNA interference-based methods for inhibiting the expression of the myotilin gene. Recombinant adeno-associated viruses of the invention deliver DNAs encoding microRNAs that knock down the expression of myotilin. The methods have application in the treatment of muscular dystrophies such as Limb Girdle Muscular Dystrophy Type 1A.



WO 2012/145597 A3

INTERNATIONAL SEARCH REPORT

International application No.
PCT/US2012/034408**A. CLASSIFICATION OF SUBJECT MATTER***C12N 7/01(2006.01)i, A61K 48/00(2006.01)i, A61K 31/7088(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)

C12N 7/01; C12N 5/06; A61K 35/76; G01M 33/53; C12N 7/00; C07K 14/00; A61K 48/00; C12N 15/861; C12N 7/02; C12N 15/85

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: myotilin, muscular dystrophy, limb girdle group, RNA interference, adeno-associated virus

C. DOCUMENTS CONSIDERED TO BE RELEVANT

Category*	Citation of document, with indication, where appropriate, of the relevant passages	Relevant to claim No.
A	US 2008-0213182 A1 (SWANSON, M. S. et al.) 04 September 2008 See the whole document.	1,2,12,13
A	US 2007-0244067 A1 (BUDKER, V. G. et al.) 18 October 2007 See the whole document.	1,2,12,13
A	US 6458929 B1 (CARPEN, O. et al.) 01 October 2002 See the whole document.	1,2,12,13
A	WO 2009-097129 A1 (APPLIED GENETIC TECHNOLOGIES CORPORATION) 06 August 2009 See the whole document.	1,2,12,13
A	US 2005-0276787 A1 (COUTO, L. B. et al.) 15 December 2005 See the whole document.	1,2,12,13
A	KR 10-2002-0028904 A (안센 과마슈티카 엔.브이.) 17 April 2002 See the whole document.	1,2,12,13
A	WO 2009-137006 A2 (THE UNIVERSITY OF NORTH CAROLINA AT CHAPEL HILL) 12 November 2009 See the whole document.	1,2,12,13

☐ 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

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INTERNATIONAL SEARCH REPORT

International application No.

PCT/US2012/034408

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. a sequence listing filed or furnished

- ☐ on paper
☒ in electronic form

b. time of filing or furnishing

- ☐ contained in the international application as filed
☒ filed together with the international application in electronic form
☐ furnished 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:

INTERNATIONAL SEARCH REPORT

International application No.

PCT/US2012/034408**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.: 3-11,14
because they relate to subject matter not required to be searched by this Authority, namely:
Said claims pertain to methods for treatment of the human body, thus relate to a subject-matter which International Searching Authority is not required to search under PCT Art. 17(2)(a)(i) and Rule 39.1(iv).
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.: 14
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.

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Information on patent family members

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