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Published:

- with international search report (Art. 21(3))
- before the expiration of the time limit for amending the claims and to be republished in the event of receipt of amendments (Rule 48.2(h))

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

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(54) Title: COMPOUNDS, COMPOSITIONS AND METHODS FOR TREATING LYSOSOMAL STORAGE DISEASES AND DISORDERS

(57) Abstract: Provided herein are lysosomotropic agents, composition and methods used to treat lysosomal-storage diseases, including ophthalmic conditions associated with the overproduction of waste products that accumulate during the course of photo-transduction.



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## INTERNATIONAL SEARCH REPORT

International application No  
PCT/US2009/037020

## A. CLASSIFICATION OF SUBJECT MATTER

INV. A61K31/13 A61K31/343 A61K31/451 A61K31/4706 A61K31/495  
A61K31/5415 A61K31/55 A61P43/00

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 A61P

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, EMBASE, 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 2007/014327 A (UNIV FLORIDA RESEARCH FOUNDAT [US]; KAUSHAL SHALESH [US]; NOORWEZ SYE) 1 February 2007 (2007-02-01)  claims 1,15	1,5-20, 22-24, 27-29, 35-37
X	WO 2007/022076 A (UAB RESEARCH FOUNDATION [US]; SHACKA JOHN J [US]; ROTH KEVIN A [US]) 22 February 2007 (2007-02-22)  claims 1,3 page 5, line 24 - page 7, line 16  -/--	1,6,9, 12,13, 16,19, 20,22, 28,35,36

 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 document 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 another 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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09/09/2009

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## INTERNATIONAL SEARCH REPORT

International application No

PCT/US2009/037020

C(Continuation). DOCUMENTS CONSIDERED TO BE RELEVANT

Category*	Citation of document, with indication, where appropriate, of the relevant passages	Relevant to claim No.
X	<p>WO 2006/116716 A (UNIV FLORIDA [US]; KAUSHAL SHALESH [US]; MALHOTRA RITU [US]; DUNN WILL) 2 November 2006 (2006-11-02)</p> <p>claims 12-17</p>	<p>1,6,9, 12,13, 16,19, 20,22, 28,35,36</p>
A	<p>A. NIEMANN ET AL: "The lysosomotropic agent monodansylcadaverine also acts as a solvent polarity probe" JOURNAL OF HISTOCHEMISTRY AND CYTOCHEMISTRY, vol. 48, no. 2, February 2000 (2000-02), pages 251-258, XP002531220 page 251, column 2, paragraph 2 - page 252, column 1, paragraph 1</p>	<p>1,5-20, 22-24, 27-37</p>
A	<p>SHAIKH N A ET AL: "AMIODARONE - AN INHIBITOR OF PHOSPHOLIPASE ACTIVITY: A COMPARATIVE STUDY OF THE INHIBITORY EFFECTS OF AMIODARONE, CHLOROQUINE AND CHLORPROMAZINE" MOLECULAR AND CELLULAR BIOCHEMISTRY, NORWELL, MA, US, vol. 76, 1 January 1987 (1987-01-01), pages 163-172, XP002935442 ISSN: 0300-8177 the whole document</p>	<p>1,5-20, 22-24, 27-37</p>

# INTERNATIONAL SEARCH REPORT

International application No.  
PCT/US2009/037020

## 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.:  
because they relate to subject matter not required to be searched by this Authority, namely:
  
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:

see additional sheet

1.  As all required additional search fees were timely paid by the applicant, this international search report covers allsearchable claims.
  
2.  As all searchable claims could be searched without effort justifying an additional fees, this Authority did not invite payment of additional fees.
  
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.:

see annex

### 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.

**FURTHER INFORMATION CONTINUED FROM PCT/ISA/ 210**

This International Searching Authority found multiple (groups of) inventions in this international application, as follows:

1. claims: 1, 5-8, 9-11 (part), 12-20, 22-24, 27-37

Use of a lysosomotropic agent to treat a lysosomal storage disease

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2. claims: 2, 3, 9-11 (part), 21, 25,

Use of a lysosomotropic agent to reduce the concentration of a toxic metabolite or of A2E in the eye

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3. claims: 4, 9-11 (part), 26

Use of a lysosomotropic agent to reduce the concentration of lipofusion in an eye

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## INTERNATIONAL SEARCH REPORT

Information on patent family members

International application No

PCT/US2009/037020

Patent document cited in search report		Publication date	Patent family member(s)	Publication date
WO 2007014327	A	01-02-2007	AU 2006272497	A1 01-02-2007
			CA 2616537	A1 01-02-2007
			CN 101267779	A 17-09-2008
			EP 1909812	A2 16-04-2008
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