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For two-letter codes and other abbreviations, refer to the "Guidance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette.

(54) Title: COMPOUNDS FOR NONSENSE SUPPRESSION, USE OF THESE COMPOUNDS FOR THE MANUFACTURE OF A MEDICAMENT FOR TREATING SOMATIC MUTATION-RELATED DISEASES

(57) Abstract: The present invention relates to compounds, compositions and their use for the manufacture of medicament for treating or preventing diseases associated with nonsense mutations in an mRNA by administering the compounds or compositions of the present invention. More particularly, the present invention relates to methods, compounds, and compositions for suppressing premature translation termination associated with a nonsense mutation in an mRNA.

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INTERNATIONAL SEARCH REPORT

International application No

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A. CLASSIFICATION OF SUBJECT MATTER

INV. A61K47/08 A61K47/16 A61K31/423 A61K31/424 A61K31/4245
A61P35/00

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 C07D

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, PAJ, EMBASE, BIOSIS, CHEM ABS Data

C. DOCUMENTS CONSIDERED TO BE RELEVANT

Category*	Citation of document, with indication, where appropriate, of the relevant passages	Relevant to claim No.
X	WO 02/36580 A (BONHAM, LYNN; LEUNG, DAVID, W; HOLLENBACK, DAVID, M; KLEIN, J., PETER;) 10 May 2002 (2002-05-10) paragraph [0022] - paragraph [0095] claims 1-30	1-32, 41-52, 55-58
X	US 2002/107269 A1 (BONHAM LYNN ET AL) 8 August 2002 (2002-08-08) paragraph [0104] - paragraph [0240] table 1 claims 1-30	1-32, 41-52, 55-58

☒ 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.
- *G* document member of the same patent family

Date of the actual completion of the international search

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Date of mailing of the international search report

08/05/2006

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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 2004/046122 A (OXFORD GLYCOSCIENCES LTD; COURTNEY, STEPHEN, MARTIN; HAY, PHILIP, AND) 3 June 2004 (2004-06-03)</p> <p>page 1, paragraph 1 - page 15, paragraph 29 examples 21,23-35,53,81-108 claims 1-16</p>	<p>1-34, 36, 37, 40-52, 55-58</p>
X	<p>DELUCA M R ET AL: "The Total Synthesis of UK-1" 13 January 1997 (1997-01-13), TETRAHEDRON LETTERS, ELSEVIER, AMSTERDAM, NL, PAGE(S) 199-202 , XP004070502 ISSN: 0040-4039 page 199</p>	<p>1-32, 41-46, 55-58</p>
X	<p>WO 99/59586 A (REGENTS OF THE UNIVERSITY OF CALIFORNIA; WANG, PING, H) 25 November 1999 (1999-11-25) claims 1-18</p>	<p>1-34</p>
X	<p>WO 98/23611 A (THE PROCTER & GAMBLE COMPANY; HENRY, RAYMOND, TODD; SHELDON, RUSSELL,) 4 June 1998 (1998-06-04) claims 1-42</p>	<p>1-34, 40-43</p>

INTERNATIONAL SEARCH REPORT

International application No.
PCT/US2005/036762

Box 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:

Although claims 1-55 are directed to a method of treatment of the human/animal body, the search has been carried out and based on the alleged effects of the compound/composition.
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 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.
- ☐ No protest accompanied the payment of additional search fees.

INTERNATIONAL SEARCH REPORT

International application No
PCT/US2005/036762

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