

PATENT COOPERATION TREATY

PCT

INTERNATIONAL SEARCH REPORT

(PCT Article 18 and Rules 43 and 44)

Applicant's or agent's file reference 8171.50.WO00	FOR FURTHER ACTION see Form PCT/ISA/220 as well as, where applicable, item 5 below.	
International application No. PCT/US2019/065581	International filing date (<i>day/month/year</i>) 11 December 2019 (11-12-2019)	(Earliest) Priority Date (<i>day/month/year</i>) 13 December 2018 (13-12-2018)
Applicant SAREPTA THERAPEUTICS, INC.		

This international search report has been prepared by this International Searching Authority and is transmitted to the applicant according to Article 18. A copy is being transmitted to the International Bureau.

This international search report consists of a total of 6 sheets.

☒ It is also accompanied by a copy of each prior art document cited in this report.

1. Basis of the report

a. With regard to the **language**, the international search was carried out on the basis of:

- ☒ the international application in the language in which it was filed
☐ a translation of the international application into _____, which is the language of a translation furnished for the purposes of international search (Rules 12.3(a) and 23.1(b))

b. ☐ This international search report has been established taking into account the **rectification of an obvious mistake** authorized by or notified to this Authority under Rule 91 (Rule 43.6**bis**(a)).

c. ☒ With regard to any **nucleotide and/or amino acid sequence** disclosed in the international application, see Box No. I.

2. ☐ **Certain claims were found unsearchable** (See Box No. II)

3. ☐ **Unity of invention is lacking** (see Box No III)

4. With regard to the **title**,

- ☒ the text is approved as submitted by the applicant
☐ the text has been established by this Authority to read as follows:

5. With regard to the **abstract**,

- ☒ the text is approved as submitted by the applicant
☐ the text has been established, according to Rule 38.2, by this Authority as it appears in Box No. IV. The applicant may, within one month from the date of mailing of this international search report, submit comments to this Authority

6. With regard to the **drawings**,

- a. the figure of the **drawings** to be published with the abstract is Figure No. _____
☐ as suggested by the applicant
☐ as selected by this Authority, because the applicant failed to suggest a figure
☐ as selected by this Authority, because this figure better characterizes the invention
- b. ☐ none of the figures is to be published with the abstract

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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:
 - a. ☒ forming part of the international application as filed:
 - ☒ in the form of an Annex C/ST.25 text file.
 - ☐ on paper or in the form of an image file.
 - b. ☐ furnished together with the international application under PCT Rule 13~~ter~~.1(a) for the purposes of international search only in the form of an Annex C/ST.25 text file.
 - c. ☐ furnished subsequent to the international filing date for the purposes of international search only:
 - ☐ in the form of an Annex C/ST.25 text file (Rule 13~~ter~~.1(a)).
 - ☐ on paper or in the form of an image file (Rule 13~~ter~~.1(b) and Administrative Instructions, Section 713).
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 forming part of the application as filed or does not go beyond the application as filed, as appropriate, were furnished.
3. Additional comments:

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A. CLASSIFICATION OF SUBJECT MATTER
INV. C12N15/113
ADD.

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

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 practicable, search terms used)

EPO-Internal, WPI 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 2017/062835 A2 (SAREPTA THERAPEUTICS INC [US]) 13 April 2017 (2017-04-13)	1-7, 9-17,19, 20,22, 25-29
A	paragraph [0276] - paragraph [0284] paragraph [0333] - paragraph [0339] claims 69-75; sequences 81, 598, 892, 1042 the whole document	8,18,21, 23,24
X	WO 2010/048586 A1 (AVI BIOPHARMA INC [US]; SAZANI PETER [US]; KOLE RYSZARD [US]) 29 April 2010 (2010-04-29)	1-7, 9-17,19, 20,22, 25-29
A	figure 3; example 2; sequences 276, 287, 585 the whole document	8,18,21, 23,24
	----- -/-	



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 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

16 April 2020

Date of mailing of the international search report

24/04/2020

Name and mailing address of the ISA/

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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	EP 2 799 548 A1 (NIPPON SHINYAKU CO LTD [JP]; NAT CT NEUROLOGY & PSYCHIATRY [JP]) 5 November 2014 (2014-11-05)	1-7, 9-17,19, 20,22, 25-29
A	claim 19; figures 26-29; examples 30-39; table 15; sequences 96, 97, 102 the whole document	8,18,21, 23,24
X	----- WU B ET AL: "Targeted skipping of human dystrophin exons in transgenic mouse model systemically for antisense drug development (art nr e19906)", PLOS ONE, PUBLIC LIBRARY OF SCIENCE, US, vol. 6, no. 5, 17 May 2011 (2011-05-17), pages 1-11, XP009171295, ISSN: 1932-6203, DOI: 10.1371/JOURNAL.PONE.0019906	1-7, 9-17,19, 20,22, 25-29
A	table 1 the whole document	8,18,21, 23,24
X	----- WO 2012/109296 A1 (CHARLOTTE MECKLENBURG HOSPITAL [US]; LU QI LONG [US]) 16 August 2012 (2012-08-16)	1-7, 9-17,19, 20,22, 25-29
A	figures 3, 4; table 3; sequences 76-99 the whole document	8,18,21, 23,24
A	----- WO 2012/150960 A1 (AVI BIOPHARMA INC [US]; HANSON GUNNAR J [US]) 8 November 2012 (2012-11-08) cited in the application figures 5-9; examples 30-32 the whole document -----	1-29

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Information on patent family members

International application No

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Patent document cited in search report		Publication date		Patent family member(s)		Publication date
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