

PATENT COOPERATION TREATY

PCT

INTERNATIONAL SEARCH REPORT

(PCT Article 18 and Rules 43 and 44)

Applicant's or agent's file reference 137510308WO1	FOR FURTHER ACTION see Form PCT/ISA/220 as well as, where applicable, item 5 below.	
International application No. PCT/US2020/042312	International filing date (<i>day/month/year</i>) 16 July 2020 (16-07-2020)	(Earliest) Priority Date (<i>day/month/year</i>) 19 July 2019 (19-07-2019)
Applicant BIOGEN MA 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 5 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 A61K31/712 A61K31/7125
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 A61K

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, BIOSIS, Sequence Search, EMBASE, WPI Data

C. DOCUMENTS CONSIDERED TO BE RELEVANT

Category*	Citation of document, with indication, where appropriate, of the relevant passages	Relevant to claim No.
A	FINKEL RICHARD S ET AL: "Treatment of infantile-onset spinal muscular atrophy with nusinersen: a phase 2, open-label, dose-escalation study", THE LANCET, ELSEVIER, AMSTERDAM, NL, vol. 388, no. 10063, 7 December 2016 (2016-12-07), pages 3017-3026, XP029849269, ISSN: 0140-6736, DOI: 10.1016/S0140-6736(16)31408-8 the whole document ----- -/--	1-30



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

6 October 2020

Date of mailing of the international search report

14/10/2020

Name and mailing address of the ISA/

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International application No
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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.
A	RICHARD S. FINKEL ET AL: "Nusinersen versus Sham Control in Infantile-Onset Spinal Muscular Atrophy", NEW ENGLAND JOURNAL OF MEDICINE, vol. 377, no. 18, 2 November 2017 (2017-11-02), pages 1723-1732, XP055528610, US ISSN: 0028-4793, DOI: 10.1056/NEJMoa1702752 the whole document	1-30
A	----- WO 2014/110291 A1 (ISIS PHARMACEUTICALS INC [US]) 17 July 2014 (2014-07-17) figure 1; examples 1-6 the whole document	1-30
A	----- WO 2016/040748 A1 (IONIS PHARMACEUTICALS INC [US]) 17 March 2016 (2016-03-17) examples 13, 14 the whole document	1-30
A	----- DERENDORF H ET AL: "Modeling of pharmacokinetic/pharmacodynamic (PK/PD) relationships: concepts and perspectives", PHARMACEUTICAL RESEARCH, SPRINGER NEW YORK LLC, US, vol. 16, no. 2, 1 February 1999 (1999-02-01), pages 176-185, XP002383973, ISSN: 0724-8741, DOI: 10.1023/A:1011907920641 the whole document	1-30
A	----- C. CIFUENTES-DIAZ ET AL: "Neurofilament accumulation at the motor endplate and lack of axonal sprouting in a spinal muscular atrophy mouse model", HUMAN MOLECULAR GENETICS, vol. 11, no. 12, 1 June 2002 (2002-06-01), pages 1439-1447, XP055574986, DOI: 10.1093/hmg/11.12.1439 the whole document -----	23-30

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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
WO 2014110291 A1	17-07-2014	EP 2943225 A1	18-11-2015
		US 2016002627 A1	07-01-2016
		US 2019040384 A1	07-02-2019
		WO 2014110291 A1	17-07-2014

WO 2016040748 A1	17-03-2016	BR 112017004056 A2	05-12-2017
		CL 2017000594 A1	01-12-2017
		CL 2019000447 A1	28-06-2019
		US 2017363643 A1	21-12-2017
		WO 2016040748 A1	17-03-2016
