

PATENT COOPERATION TREATY

PCT

INTERNATIONAL SEARCH REPORT

(PCT Article 18 and Rules 43 and 44)

Applicant's or agent's file reference 28335/52684/PC	FOR FURTHER ACTION see Form PCT/ISA/220 as well as, where applicable, item 5 below.	
International application No. PCT/US2019/039893	International filing date (<i>day/month/year</i>) 28 June 2019 (28-06-2019)	(Earliest) Priority Date (<i>day/month/year</i>) 29 June 2018 (29-06-2018)
Applicant RESEARCH INSTITUTE AT NATIONWIDE CHILDREN'S...		

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

INTERNATIONAL SEARCH REPORT

International application No.

PCT/US2019/039893

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:

INTERNATIONAL SEARCH REPORT

International application No
PCT/US2019/039893

A. CLASSIFICATION OF SUBJECT MATTER
INV. C12N15/864 C12N9/64 A61K48/00
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 C07K 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, 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.
X	US 2016/058890 A1 (BUJ BELLO ANA MARIA [FR] ET AL) 3 March 2016 (2016-03-03)	1,11-13, 16-19, 23-25, 29-31, 35,36
Y	paragraphs [0069], [0085], [0178], [0179], [0217]; claims 1-16; figure 1 ----- -/--	2-10,14, 20-22, 26-28, 32-34



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 September 2019

Date of mailing of the international search report

25/09/2019

Name and mailing address of the ISA/

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INTERNATIONAL SEARCH REPORT

International application No

PCT/US2019/039893

C(Continuation). DOCUMENTS CONSIDERED TO BE RELEVANT

Category*	Citation of document, with indication, where appropriate, of the relevant passages	Relevant to claim No.
X	ROUDAUT CARINNE ET AL: "Restriction of Calpain3 Expression to the Skeletal Muscle Prevents Cardiac Toxicity and Corrects Pathology in a Murine Model of Limb-Girdle Muscular Dystrophy", 3 September 2013 (2013-09-03), CIRCULATION, VOL. 128, NR. 10, PAGE(S) 1094-1104, XP002794062, ISSN: 0009-7322(print)	1,11,12, 15-19, 23-25, 29-31, 35,36
Y	the whole document	2-10,13, 14, 20-22, 26-28, 32-34
X	----- DAN WANG ET AL: "The potential of adeno-associated viral vectors for gene delivery to muscle tissue", EXPERT OPINION ON DRUG DELIVERY, vol. 11, no. 3, 1 March 2014 (2014-03-01), pages 345-364, XP055232689, GB ISSN: 1742-5247, DOI: 10.1517/17425247.2014.871258	1,11-13, 16-19, 23-25, 29-31, 35,36
Y	page 6, paragraph 4 - page 9, paragraph 1	2-10,14, 15, 20-22, 26-28, 32-34
Y	----- RICHARD I ET AL: "MUTATIONS IN THE PROTEOLYTIC ENZYME CALPAIN 3 CAUSE LIMB-GIRDLE MUSCULAR DYSTROPHY TYPE 2A", CELL, ELSEVIER, AMSTERDAM, NL, vol. 81, no. 1, 7 April 1995 (1995-04-07), pages 27-40, XP002010548, ISSN: 0092-8674, DOI: 10.1016/0092-8674(95)90368-2 figure 2	2-10
Y	----- WO 2017/180976 A1 (RES INST AT NATIONWIDE CHILDREN'S HOSPITAL [US]) 19 October 2017 (2017-10-19) paragraph [0092]; sequence 6 ----- -/--	13-15

INTERNATIONAL SEARCH REPORT

International application No
PCT/US2019/039893

C(Continuation). DOCUMENTS CONSIDERED TO BE RELEVANT		
Category*	Citation of document, with indication, where appropriate, of the relevant passages	Relevant to claim No.
Y	LOUISE R. RODINO-KLAPAC ET AL: "Micro-dystrophin and follistatin co-delivery restores muscle function in aged DMD model", HUMAN MOLECULAR GENETICS, vol. 22, no. 24, 1 January 2013 (2013-01-01), pages 4929-4937, XP055478537, gb ISSN: 0964-6906, DOI: 10.1093/hmg/ddt342 page 4935, column 1, paragraph 3 - page 4935, column 2, paragraph 2 -----	8-10
Y	WO 2017/087395 A1 (RES INST AT NATIONWIDE CHILDREN'S HOSPITAL [US]) 26 May 2017 (2017-05-26) examples 2, 3 -----	8-10

INTERNATIONAL SEARCH REPORT

Information on patent family members

International application No

PCT/US2019/039893

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		WO 2017087395 A1	26-05-2017
