

PATENT COOPERATION TREATY

PCT

INTERNATIONAL SEARCH REPORT

(PCT Article 18 and Rules 43 and 44)

Applicant's or agent's file reference PF0314-WO-PCT	FOR FURTHER ACTION see Form PCT/ISA/220 as well as, where applicable, item 5 below.	
International application No. PCT/EP2022/080020	International filing date (<i>day/month/year</i>) 26 October 2022 (26-10-2022)	(Earliest) Priority Date (<i>day/month/year</i>) 28 October 2021 (28-10-2021)
Applicant UCB BIOPHARMA SRL		

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 8 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 43.6bis(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.
 - b. ☐ furnished subsequent to the international filing date for the purposes of international search (Rule 13*ter*.1(a)).

☐ accompanied by a statement to the effect that the sequence listing does not go beyond the disclosure in the international application as filed.
2. ☐ With regard to any nucleotide and/or amino acid sequence disclosed in the international application, this report has been established to the extent that a meaningful search could be carried out without a WIPO Standard ST.26 compliant sequence listing.
3. Additional comments:

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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 all searchable 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.:

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.

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A. CLASSIFICATION OF SUBJECT MATTER INV. C12N15/86 A61P1/00 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 A61P C07K		
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	WO 2018/187363 A1 (ENCODED THERAPEUTICS INC [US]) 11 October 2018 (2018-10-11)	1, 4-16
Y	claims; examples; sequences 40 -----	1-16
X	WO 2010/020642 A1 (BRAINCO BIOPHARMA S L [ES]; GUERRERO MARTINEZ MARIA JOSE [ES] ET AL.) 25 February 2010 (2010-02-25)	1-3, 5
Y	claims; examples; sequences -----	1-16
X	WO 2009/084472 A1 (PUBLIC UNIVERSITY CORP YOKOHAM [JP]; MATSUMOTO NAOMICHI [JP] ET AL.) 9 July 2009 (2009-07-09)	1-3, 5
Y	claims; examples; sequences -----	1-16
X, P	WO 2022/011390 A1 (BAYLOR COLLEGE MEDICINE [US]) 13 January 2022 (2022-01-13)	1-16
	claims; examples; sequences ----- -/-	
☒ 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		Date of mailing of the international search report
16 February 2023		24/02/2023
Name and mailing address of the ISA/ European Patent Office, P.B. 5818 Patentlaan 2 NL - 2280 HV Rijswijk Tel. (+31-70) 340-2040, Fax: (+31-70) 340-3016		Authorized officer Sommer, Birgit

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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 2022/214635 A1 (STICHTING VU [NL]) 13 October 2022 (2022-10-13) claims; examples; sequences -----	1-16
Y	WO 2021/089856 A1 (HORAMA [FR]) 14 May 2021 (2021-05-14) page 32, line 1 - line 16; claims; examples -----	1-16

INTERNATIONAL SEARCH REPORT

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 2018187363 A1	11-10-2018	AU 2018250161 A1 BR 112019020777 A2 CA 3058189 A1 CL 2019002805 A1 CN 110730823 A CO 2019011450 A2 EA 201992358 A1 EP 3607073 A1 IL 269767 A JP 2020515292 A KR 20190137126 A SG 11201909203W A TW 201842185 A US 2019024118 A1 US 2019024119 A1 US 2019024120 A1 US 2019024121 A1 US 2020165628 A1 WO 2018187363 A1	17-10-2019 28-04-2020 11-10-2018 06-03-2020 24-01-2020 31-10-2019 24-03-2020 12-02-2020 28-11-2019 28-05-2020 10-12-2019 28-11-2019 01-12-2018 24-01-2019 24-01-2019 24-01-2019 24-01-2019 28-05-2020 11-10-2018
WO 2010020642 A1	25-02-2010	EP 2321417 A1 ES 2546732 T3 US 2011268747 A1 WO 2010020642 A1	18-05-2011 28-09-2015 03-11-2011 25-02-2010
WO 2009084472 A1	09-07-2009	JP 5608863 B2 JP WO2009084472 A1 WO 2009084472 A1	15-10-2014 19-05-2011 09-07-2009
WO 2022011390 A1	13-01-2022	NONE	
WO 2022214635 A1	13-10-2022	NONE	
WO 2021089856 A1	14-05-2021	AU 2020378643 A1 CA 3160619 A1 CN 114929869 A EP 4055151 A1 IL 292814 A KR 20220094216 A US 2021162072 A1 US 2022323610 A1 WO 2021089856 A1	02-06-2022 14-05-2021 19-08-2022 14-09-2022 01-07-2022 05-07-2022 03-06-2021 13-10-2022 14-05-2021

FURTHER INFORMATION CONTINUED FROM PCT/ISA/ 210

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

1. claims: 1-16

a nucleic acid construct comprising a transgene encoding a syntaxin binding protein 1 (STXBP1) as well as subject-matter related thereto;

1.1. claims: 1-16 (partially)

a nucleic acid construct comprising a transgene encoding a STXBP1 having SEQ ID NO: 9 as well as subject-matter related thereto;

1.2. claims: 1-16 (partially)

a nucleic acid construct comprising a transgene encoding a STXBP1 having SEQ ID NO: 10 as well as subject-matter related thereto;

1.3. claims: 1-16 (partially)

a nucleic acid construct comprising a transgene encoding a STXBP1 having SEQ ID NO: 11 as well as subject-matter related thereto;

1.4. claims: 1-16 (partially)

a nucleic acid construct comprising a transgene encoding a STXBP1 having SEQ ID NO: 12 as well as subject-matter related thereto;

1.5. claims: 1-16 (partially)

a nucleic acid construct comprising a transgene encoding a STXBP1 having SEQ ID NO: 13 as well as subject-matter related thereto;

1.6. claims: 1-16 (partially)

a nucleic acid construct comprising a transgene encoding a STXBP1 having SEQ ID NO: 14 as well as subject-matter related thereto;

1.7. claims: 1-16 (partially)

a nucleic acid construct comprising a transgene encoding a STXBP1 having SEQ ID NO: 15 as well as subject-matter related thereto;

1.8. claims: 1-16 (partially)

a nucleic acid construct comprising a transgene encoding a STXBP1 having SEQ ID NO: 16 as well as subject-matter

FURTHER INFORMATION CONTINUED FROM PCT/ISA/ 210

related thereto;
