

(12) INTERNATIONAL APPLICATION PUBLISHED UNDER THE PATENT COOPERATION TREATY (PCT)

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
29 June 2023 (29.06.2023)



(10) International Publication Number
WO 2023/122689 A2

(51) International Patent Classification:

A61K 38/17 (2006.01) A61P 35/00 (2006.01)
C07K 14/47 (2006.01) A61P 37/02 (2006.01)
C12N 15/10 (2006.01) C12N 9/64 (2006.01)

(21) International Application Number:

PCT/US2022/082177

(22) International Filing Date:

21 December 2022 (21.12.2022)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

63/293,040 22 December 2021 (22.12.2021) US

(71) Applicant: VERTEX PHARMACEUTICALS INCORPORATED [US/US]; 50 Northern Avenue, Boston, Massachusetts 02210 (US).

(72) Inventors: BLOUSE, Grant E.; c/o Catalyst Biosciences, Inc., 611 Gateway Boulevard, Suite 710, South San Francisco, California 94080 (US). JENSEN, Jan Kristian; c/

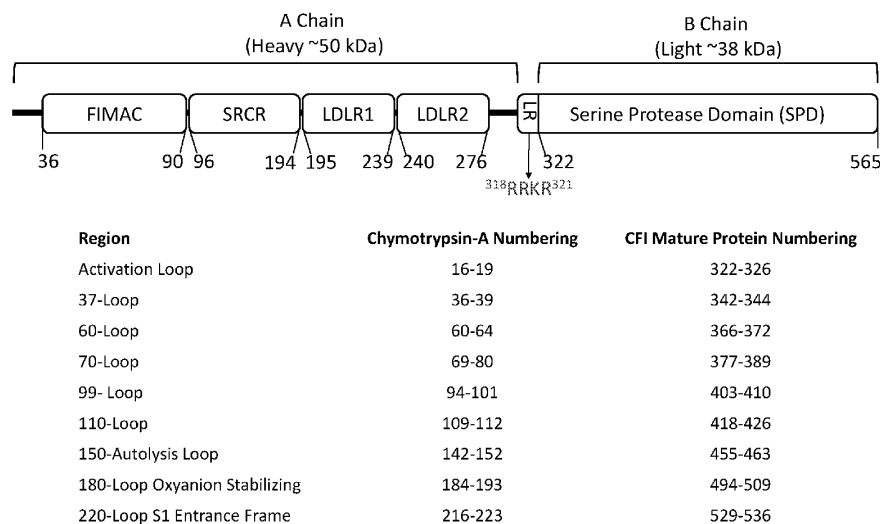
o Aarhus University, Nordre Ringvej 1, 8000 Aarhus C (DK). OLDENBURG, Emil; c/o Aarhus University, Nordre Ringvej 1, 8000 Aarhus C (DK). SCHAR, Christine René; c/o Aarhus University, Nordre Ringvej 1, 8000 Aarhus C (DK). JENDROSZEK, Agnieszka; c/o Aarhus University, Nordre Ringvej 1, 8000 Aarhus C (DK). MCGUIRE, James N.; c/o Catalyst Biosciences, Inc., 611 Gateway Boulevard, Suite 710, South San Francisco, California 94080 (US). IYER, Shyam Rajan; c/o Catalyst Biosciences, Inc., 611 Gateway Boulevard, Suite 710, South San Francisco, California 94080 (US). PELOT, Kyle A.; c/o Catalyst Biosciences, Inc., 611 Gateway Boulevard, Suite 710, South San Francisco, California 94080 (US).

(74) Agent: MIHELICIC, John M. et al.; Cooley LLP, 1299 Pennsylvania Ave., Suite 700, Washington, District of Columbia 20004 (US).

(81) Designated States (unless otherwise indicated, for every kind of national protection available): AE, AG, AL, AM, AO, AT, AU, AZ, BA, BB, BG, BH, BN, BR, BW, BY, BZ, CA, CH, CL, CN, CO, CR, CU, CV, CZ, DE, DJ, DK, DM, DO, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, GT,

(54) Title: COMPLEMENT FACTOR I-RELATED COMPOSITIONS AND METHODS

FIG. 1



(57) Abstract: Provided herein are Complement Factor I (CFI) variants and CFI containing fusion constructs that exhibit at least one improved characteristic relative to a wild type CFI. The CFI variants and fusion constructs of the disclosure can exhibit tunable specificity and activity. The CFI variants and fusion constructs provided herein may be useful for treating a disease or condition associated with dysregulation of the complement system or a deficiency of CFI.

[Continued on next page]

HN, HR, HU, ID, IL, IN, IQ, IR, IS, IT, JM, JO, JP, KE, KG, KH, KN, KP, KR, KW, KZ, LA, LC, LK, LR, LS, LU, LY, MA, MD, MG, MK, MN, MW, MX, MY, MZ, NA, NG, NI, NO, NZ, OM, PA, PE, PG, PH, PL, PT, QA, RO, RS, RU, RW, SA, SC, SD, SE, SG, SK, SL, ST, SV, SY, TH, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, WS, ZA, ZM, ZW.

(84) Designated States (*unless otherwise indicated, for every kind of regional protection available*): ARIPO (BW, CV, GH, GM, KE, LR, LS, MW, MZ, NA, RW, SD, SL, ST, SZ, TZ, UG, ZM, ZW), Eurasian (AM, AZ, BY, KG, KZ, RU, TJ, TM), European (AL, AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HR, HU, IE, IS, IT, LT, LU, LV, MC, ME, MK, MT, NL, NO, PL, PT, RO, RS, SE, SI, SK, SM, TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, KM, ML, MR, NE, SN, TD, TG).

Declarations under Rule 4.17:

- *as to applicant's entitlement to apply for and be granted a patent (Rule 4.17(ii))*
- *as to the applicant's entitlement to claim the priority of the earlier application (Rule 4.17(iii))*

Published:

- *without international search report and to be republished upon receipt of that report (Rule 48.2(g))*
- *with sequence listing part of description (Rule 5.2(a))*