

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
20 April 2023 (20.04.2023)



(10) International Publication Number
WO 2023/064858 A1

(51) International Patent Classification:

A61K 39/395 (2006.01) C12N 15/113 (2010.01)
C12N 9/22 (2006.01)

(21) International Application Number:

PCT/US2022/078050

(22) International Filing Date:

13 October 2022 (13.10.2022)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

63/255,290 13 October 2021 (13.10.2021) US

(71) Applicants: APELLIS PHARMACEUTICALS, INC.

[US/US]; 100 5th Avenue, Waltham, Massachusetts 02451 (US). BEAM THERAPEUTICS, INC. [US/US]; 238 Main Street, 9th Floor, Cambridge, Massachusetts 02142 (US).

(72) Inventors; and

(71) Applicants: JOHNSON, Lei Wang [US/US]; c/o Beam Therapeutics, Inc., 238 Main Street, 9th Floor, Cam-

bridge, Massachusetts 02142 (US). BOHNUUD, Tanggis [US/US]; c/o Beam Therapeutics, Inc., 238 Main Street, 9th Floor, Cambridge, Massachusetts 02142 (US).

(72) Inventors: FRANCOIS, Cedric; c/o Apellis Pharmaceuticals, Inc., 100 5th Avenue, Waltham, Massachusetts 02451 (US). KOLEV, Martin; c/o Apellis Pharmaceuticals, Inc., 100 5th Avenue, Waltham, Massachusetts 02451 (US).

(74) Agent: LIVINGSTON, Jennifer L. et al.; Dinsmore & Shohl LLP, 255 East Fifth Street, Suite 1900, Cincinnati, Ohio 45202 (US).

(74) Agent: LIVINGSTON, Jennifer L. et al.; Dinsmore & Shohl LLP, 255 East Fifth Street, Suite 1900, Cincinnati, Ohio 45202 (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, 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,

(54) Title: COMPOSITIONS AND METHODS FOR GENOME EDITING THE NEONATAL FC RECEPTOR

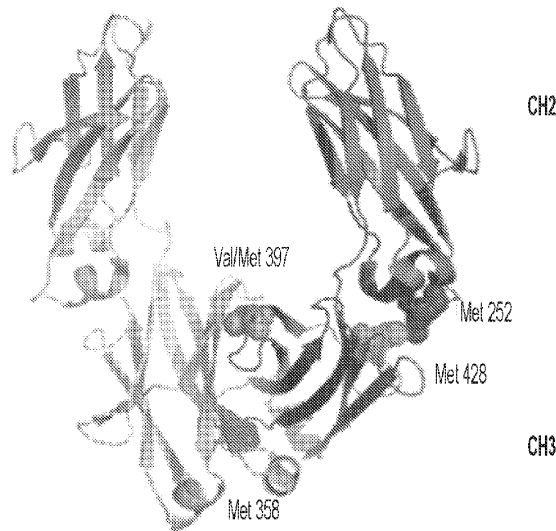


FIG. 2B

(57) Abstract: Provided herein are compositions and methods for modifying the gene encoding a neonatal fragment crystallizable receptor (FcRn) protein and/or expression or activity thereof in a mammalian cell. The compositions and methods disclosed herein provide variant FcRn proteins having reduced ability to bind to an Fc region of an IgG antibody.

[Continued on next page]

WO 2023/064858 A1

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, 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:

- *with international search report (Art. 21(3))*
- *before the expiration of the time limit for amending the claims and to be republished in the event of receipt of amendments (Rule 48.2(h))*
- *with sequence listing part of description (Rule 5.2(a))*