

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

(19) World Intellectual Property
Organization

International Bureau

(43) International Publication Date
03 February 2022 (03.02.2022)



(10) International Publication Number
WO 2022/023761 A2

(51) International Patent Classification:

C12N 9/24 (2006.01) A61K 38/47 (2006.01)

(21) International Application Number:

PCT/GB2021/051969

(22) International Filing Date:

29 July 2021 (29.07.2021)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

2011813.9	29 July 2020 (29.07.2020)	GB
2100648.1	18 January 2021 (18.01.2021)	GB
2105924.1	26 April 2021 (26.04.2021)	GB

(71) Applicant: **FREELINE THERAPEUTICS LIMITED**

[GB/GB]; Stevenage Bioscience Catalyst, Gunnels Wood Road, Stevenage Hertfordshire SG1 2FX (GB).

(72) Inventors: **COMPER, Fabrizio**; c/o Freeline Therapeutics

Limited, Stevenage Bioscience Catalyst, Gunnels Wood Road, Stevenage, Hertfordshire, SG1 2FX (GB). **NATH-WANI, Amit**; c/o Freeline Therapeutics Limited, Stevenage Bioscience Catalyst, Gunnels Wood Road, Stevenage, Hertfordshire, SG1 2FX (GB). **MCINTOSH, Jenny**; c/o Freeline Therapeutics Limited, Stevenage Bioscience Catalyst, Gunnels Wood Road, Stevenage, Hertfordshire, SG1 2FX (GB). **CORBAU, Romuald**; c/o Freeline Therapeutics Limited, Stevenage Bioscience Catalyst, Gunnels Wood Road, Stevenage, Hertfordshire, SG1 2FX (GB). **KIA, Azadeh**; c/o Freeline Therapeutics Limited, Stevenage Bioscience Catalyst, Gunnels Wood Road, Stevenage, Hertfordshire, SG1 2FX (GB). **MIRANDA, Carlos**; c/o Freeline Therapeutics Limited, Stevenage Bioscience Catalyst, Gunnels Wood Road, Stevenage, Hertfordshire, SG1 2FX (GB).

(74) Agent: **J A KEMP LLP**; 80 Turnmill Street, London

EC1M 5QU (GB).

(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, CZ, DE, DJ, DK, DM, DO, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, GT, HN, HR, HU, ID, IL, IN, IR, IS, IT, JO, JP, KE, KG, KH, KN, KP, KR, KW, KZ, LA, LC, LK, LR, LS, LU, LY, MA, MD,

ME, 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, 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, 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))
- of inventorship (Rule 4.17(iv))

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))

(54) Title: POLYPEPTIDE

(57) Abstract: The present invention relates to a modified β -Glucocerebrosidase (GCase) polypeptide and a polynucleotide comprising a modified glucocerebrosidase (GBA) nucleotide sequence. The invention further relates to a viral particle comprising a recombinant genome comprising the polynucleotide of the invention, and a composition comprising the modified GCase polypeptide, polynucleotide, or viral particle of the invention. The invention also relates to methods of using, and uses of, the modified GCase polypeptide, polynucleotide, viral particle and/or composition of the invention. The invention further relates to the modified GCase polypeptide, polynucleotide, viral particle, or composition of the invention for use in a method of treatment, or use in the manufacture of a medicament for use in a method of treatment.



WO 2022/023761 A2