

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
24 February 2022 (24.02.2022)



(10) International Publication Number
WO 2022/040239 A1

(51) International Patent Classification:

C07K 14/47 (2006.01) C12N 15/864 (2006.01)
A61K 48/00 (2006.01)

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.

(21) International Application Number:

PCT/US2021/046382

(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).

(22) International Filing Date:

17 August 2021 (17.08.2021)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

63/066,570 17 August 2020 (17.08.2020) US

(71) Applicant: MASSACHUSETTS INSTITUTE OF
TECHNOLOGY [US/US]; 77 Massachusetts Avenue,
Cambridge, MA 02139 (US).

(72) Inventors: FENG, Guoping; C/o The Broad Institute, Inc.,
415 Main Street, Cambridge, MA 02142 (US). GAO, Xiao;
C/o The Broad Institute, Inc., 415 Main Street, Cambridge,
MA 02142 (US). MEI, Yuan; C/o The Broad Institute, Inc.,
415 Main Street, Cambridge, MA 02142 (US).

(74) Agent: TAN, Shirley et al.; Wolf, Greenfield & Sacks,
P.C., 600 Atlantic Avenue, Boston, MA 02210-2206 (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, 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,

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))

(54) Title: SHANK3 GENE THERAPY APPROACHES

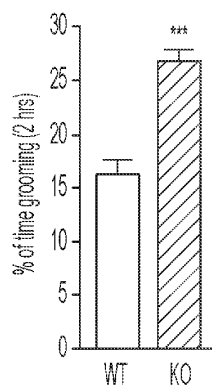


FIG. 1B

(57) Abstract: Aspects of the disclosure relate to non-naturally occurring polynucleotides encoding a Shank3 protein, AAV vectors comprising the polynucleotides, and gene therapy methods.

WO 2022/040239 A1