

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
19 January 2023 (19.01.2023)



(10) International Publication Number
WO 2023/287861 A2

(51) International Patent Classification:

A61K 48/00 (2006.01) A61K 47/69 (2017.01)

(21) International Application Number:

PCT/US2022/036930

(22) International Filing Date:

13 July 2022 (13.07.2022)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

63/221,290 13 July 2021 (13.07.2021) US

(71) Applicant: **GENERATION BIO CO.** [US/US]; 301 Binney Street, 4th Floor, Cambridge, Massachusetts 02142 (US).

(72) Inventors: **SAMAYOA, Phillip**; c/o Generation Bio Co., 301 Binney Street, 4th Floor, Cambridge, Massachusetts 02142 (US). **SILVER, Nathaniel**; c/o Generation Bio Co., 301 Binney Street, 4th Floor, Cambridge, Massachusetts 02142 (US). **LI, Prudence, Yui Tung**; c/o Generation Bio Co., 301 Binney Street, 4th Floor, Cambridge, Massachusetts 02142 (US). **TOY, Randall, Newton**; c/o Generation Bio Co., 301 Binney Street, 4th Floor, Cambridge, Massachusetts 02142 (US). **NOLTING, Birte**; c/o Generation Bio Co., 301 Binney Street, 4th Floor, Cambridge, Massachusetts 02142 (US). **OONTHONPAN, Lalita**; c/o Generation Bio Co., 301 Binney Street, 4th Floor, Cambridge, Massachusetts 02142 (US).

(74) Agent: **CLARKE, Marcie B.** et al.; McCarter & English, LLP, 265 Franklin Street, Boston, Massachusetts 02110 (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, 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))
- 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))



WO 2023/287861 A2

(54) Title: SINGLE CHAIN VARIABLE FRAGMENT (SCFV) MODIFIED LIPID NANOPARTICLE COMPOSITIONS AND USES THEREOF

(57) Abstract: Provided herein are pharmaceutical compositions comprising a lipid nanoparticle (LNP) and a therapeutic nucleic acid (TNA), wherein the LNP comprises a single-chain variable fragment (scFv) linked to the LNP, and at least one pharmaceutically acceptable excipient. The scFv is capable of binding an antigen present on the surface of a cell, advantageously providing LNP compositions that target only those cells or tissues expressing the receptor.