

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
19 October 2023 (19.10.2023)



(10) International Publication Number
WO 2023/201276 A2

(51) International Patent Classification:
A61K 47/69 (2017.01) *A61K 38/46* (2006.01)

SI, SK, SM, TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, KM, ML, MR, NE, SN, TD, TG).

(21) International Application Number:
PCT/US2023/065691

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))

(22) International Filing Date:
12 April 2023 (12.04.2023)

(25) Filing Language: English

(26) Publication Language: English

Published:

- without international search report and to be republished upon receipt of that report (Rule 48.2(g))

(30) Priority Data:
63/362,858 12 April 2022 (12.04.2022) US
63/334,476 25 April 2022 (25.04.2022) US
63/342,471 16 May 2022 (16.05.2022) US
63/495,461 11 April 2023 (11.04.2023) US

(71) Applicant: **ORTHO BIO THERAPEUTICS, INC.**
[US/US]; 1300 N. Frontage Road W., Box 5937, Vail, CO 81657 (US).

(72) Inventors; and

(71) Applicants: **ALLEN, Matthew J.** [GB/GB]; 33 High Street, Swaffham Bulbeck, Cambs CB25 0HP (GB). **GEN-TSCH, George** [CH/GB]; 12 Lents Way, Cambridge CB4 1UA (GB).

(72) Inventor: **MILLETT, Peter J.**; 975 Pilgrim Drive, Edwards, CO 81632 (US).

(74) Agent: **GHIDU, Victor P.** et al.; Morgan Lewis & Bockius LLP, 1701 Market Street, Philadelphia, PA 19103 (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, MU, 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, SC, 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,

(54) Title: LIPID NANOPARTICLES FOR GENE EDITING SYSTEMS

(57) Abstract: The present disclosure provides compositions and methods for treating and preventing localized nociception, inflammation, or morphological changes associated with joint disease or illness, back or spine conditions or disorders, and musculoskeletal diseases or dysfunction with an LNP-encapsulated CRISPR/Cas9 gene editing system.

WO 2023/201276 A2