

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
09 February 2023 (09.02.2023)



(10) International Publication Number
WO 2023/014938 A1

(51) International Patent Classification:

C07H 15/26 (2006.01) C07D 307/20 (2006.01)
A61K 31/70 (2006.01) C07D 201/00 (2006.01)
C07F 9/00 (2006.01)

(21) International Application Number:

PCT/US2022/039517

(22) International Filing Date:

05 August 2022 (05.08.2022)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

63/229,628 05 August 2021 (05.08.2021) US

(71) Applicant: SANEGENE BIO USA INC. [US/US]; 1 Broadway, FL 14, Cambridge, Massachusetts 02142 (US).

(72) Inventors: WANG, Weimin; 1 Broadway, FL 14, Cambridge, Massachusetts 02142 (US). CAI, Xiaochuan; 1 Broadway, FL 14, Cambridge, Massachusetts 02142 (US).

(74) Agent: CHEN, Chen et al.; 1299 Pennsylvania Ave., NW Suite 700, Washington, District of Columbia 20004 (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,

(54) Title: 1'-ALKYL MODIFIED RIBOSE DERIVATIVES AND METHODS OF USE

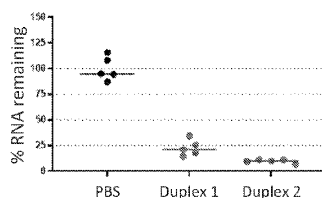
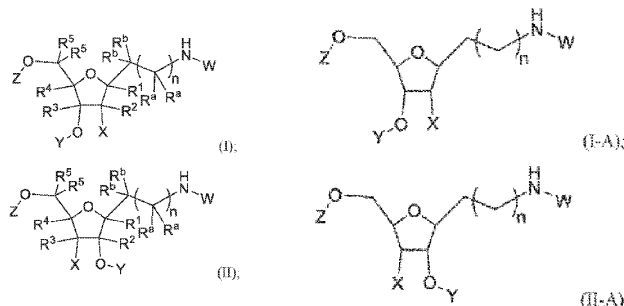


FIG. 1

(57) Abstract: The present disclosure provides linker compounds of Formula (I) or (II) pharmaceutically acceptable salts thereof, and related scaffolds and conjugates. More specifically, linker compounds of formulas (I-A), (II-A) are provided: The present disclosure also relates to uses of the linker compounds, scaffolds, and conjugates, e.g., in delivering nucleic acid and/or treating or preventing diseases.

[Continued on next page]

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))*

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))*