

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
16 February 2023 (16.02.2023)



(10) International Publication Number
WO 2023/016999 A2

(51) International Patent Classification:

C07H 15/04 (2006.01) C07H 21/04 (2006.01)
C07H 15/203 (2006.01) A61K 47/54 (2017.01)
C07H 19/06 (2006.01) C12N 15/11 (2006.01)
C07H 21/02 (2006.01)

(21) International Application Number:

PCT/EP2022/072271

(22) International Filing Date:

08 August 2022 (08.08.2022)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

2111456.6 09 August 2021 (09.08.2021) GB

(71) Applicant: **CARGENE THERAPEUTICS PTE. LTD.**
[SG/SG]; 3 Biopolis Drive, Synapse, #06-11, 138623 (SG).

(71) Applicant (for LS only): **CLEGG, Richard** [GB/GB]; c/o
Mewburn Ellis LLP, Aurora Building, Counterslip, Bristol
BS1 6BX (GB).

(72) Inventors: **LIN, Xi**; c/o Cargene Therapeutics Pte. Ltd.,
3 Biopolis Drive, Synapse, #06-11, 138623 (SG). **YUAN
HAN, Bing**; c/o Cargene Therapeutics Pte. Ltd., 3 Biopo-
lis Drive, Synapse, #06-11, 138623 (SG). **KOON EE, Pin**;

c/o Cargene Therapeutics Pte. Ltd., 3 Biopolis Drive, Sy-
napse, #06-11, 138623 (SG). **YANG, Binxia**; c/o Cargene
Therapeutics Pte. Ltd., 3 Biopolis Drive, Synapse, #06-11,
138623 (SG). **LENG LOW, Joo**; c/o Cargene Therapeutics
Pte. Ltd., 3 Biopolis Drive, Synapse, #06-11, 138623 (SG).

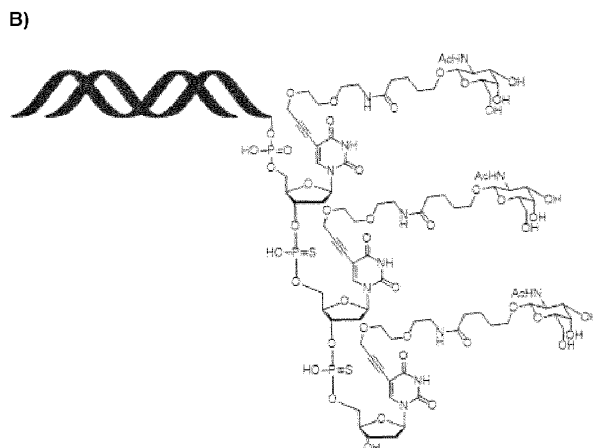
(74) Agent: **MEWBURN ELLIS LLP**; Aurora Building, Coun-
terslip, Bristol BS1 6BX (GB).

(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, 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,

(54) Title: Compounds

Figure 8



(57) Abstract: GalNAc monomers and GalNAc-oligonucleotide conjugates made using said GalNAc monomers, for example, GalNAc-bearing nucleic acids, e.g. sa-RNAs and siRNAs that may be useful in regulating expression of a target gene.

[Continued on next page]

WO 2023/016999 A2

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:

— *of inventorship (Rule 4.17(iv))*

Published:

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