

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
30 January 2025 (30.01.2025)



(10) International Publication Number
WO 2025/021034 A1

(51) International Patent Classification:

C12N 15/113 (2010.01) A61K 31/713 (2006.01)

(21) International Application Number:

PCT/CN2024/106419

(22) International Filing Date:

19 July 2024 (19.07.2024)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

202310900474.0	21 July 2023 (21.07.2023)	CN
202310904516.8	21 July 2023 (21.07.2023)	CN
202311532296.7	16 November 2023 (16.11.2023)	CN
202410396475.0	02 April 2024 (02.04.2024)	CN

(71) Applicant: **VISIRNA THERAPEUTICS (SUZHOU) CO., LTD.** [CN/CN]; Unit 401, Building 5, Block B, Phase III, Biomedical Industrial Park, No. 99 Jingu Road, Suzhou Area, China (Jiangsu) Pilot Free Trade Zone, Suzhou, Jiangsu 215028 (CN).

(72) Inventors: **ZOU, Xiaoming**; Unit 401, Building 5, Block B, Phase III, Biomedical Industrial Park, No. 99 Jingu Road, Suzhou Area, China (Jiangsu) Pilot Free Trade Zone, Suzhou, Jiangsu 215028 (CN). **LU, Jianyu**; Unit 401, Building 5, Block B, Phase III, Biomedical Industrial Park, No. 99 Jingu Road, Suzhou Area, China (Jiangsu) Pilot Free Trade Zone, Suzhou, Jiangsu 215028 (CN). **LI, Qingfeng**; Unit 401, Building 5, Block B, Phase III, Biomedical Industrial Park, No. 99 Jingu Road, Suzhou Area, China (Jiangsu) Pilot Free Trade Zone, Suzhou, Jiangsu 215028 (CN). **GAO, Cuicui**; Unit 401, Building 5, Block B, Phase III, Biomedical Industrial Park, No. 99 Jingu Road, Suzhou Area, China (Jiangsu) Pilot Free Trade Zone, Suzhou, Jiangsu 215028 (CN). **HE, Qionger**; Unit 401, Building 5, Block B, Phase III, Biomedical Industrial Park, No. 99 Jingu Road, Suzhou Area, China (Jiangsu) Pilot Free Trade Zone, Suzhou, Jiangsu 215028 (CN). **YANG, Hongkuan**; Unit 401, Building 5, Block B, Phase III, Biomedical Industrial Park, No. 99 Jingu Road, Suzhou Area, China (Jiangsu) Pilot Free Trade Zone, Suzhou, Jiangsu 215028 (CN). **WANG, Mingjie**; Unit 401, Building 5, Block B, Phase III, Biomedical Industrial Park, No. 99 Jingu Road, Suzhou Area, China (Jiangsu) Pilot Free Trade Zone, Suzhou, Jiangsu 215028 (CN).

(74) Agent: **JUN HE LAW OFFICES**; 20/F, China Resources Building 8 Jianguomenbei Avenue, Dongcheng District, Beijing 100005 (CN).

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

Published:

- with international search report (Art. 21(3))
- with sequence listing part of description (Rule 5.2(a))

(54) Title: DOUBLE-STRANDED OLIGONUCLEOTIDE AGENTS AND USES THEREOF

(57) Abstract: Provided are a double-stranded oligonucleotide agent and uses. The double-stranded oligonucleotide agent comprises a specific cleavage site for an endonuclease, wherein the cleaved double-stranded oligonucleotide agent is capable of inhibiting expression of the target gene via RNA interference (RNAi).



WO 2025/021034 A1