

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
18 November 2021 (18.11.2021)



(10) International Publication Number
WO 2021/231107 A1

(51) International Patent Classification:

C12N 15/113 (2010.01) A61K 31/7125 (2006.01)

C12N 9/14 (2006.01) A61P 27/02 (2006.01)

(21) International Application Number:

PCT/US2021/030254

(22) International Filing Date:

30 April 2021 (30.04.2021)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

63/023,013 11 May 2020 (11.05.2020) US

63/112,458 11 November 2020 (11.11.2020) US

(71) Applicant: **STOKE THERAPEUTICS, INC.** [US/US];
45 Wiggins Avenue, Bedford, MA 01730 (US).

(72) Inventors: **AZNAREZ, Isabel**; 45 Wiggins Avenue, Bedford, MA 01730 (US). **VENKATESH, Aditya**; 45 Wiggins Avenue, Bedford, MA 01730 (US). **LIAU, Gene**; 45 Wiggins Avenue, Bedford, MA 01730 (US).

(74) Agent: **HUANG, Wei**; Wilson Sonsini Goodrich & Rosati,
650 Page Mill Road, Palo Alto, CA 94304 (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, CZ, DE, DJ, DK, DM, DO, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, GT, HN, HR, HU, ID, IL, IN, IR, IS, IT, 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: OPA1 ANTISENSE OLIGOMERS FOR TREATMENT OF CONDITIONS AND DISEASES

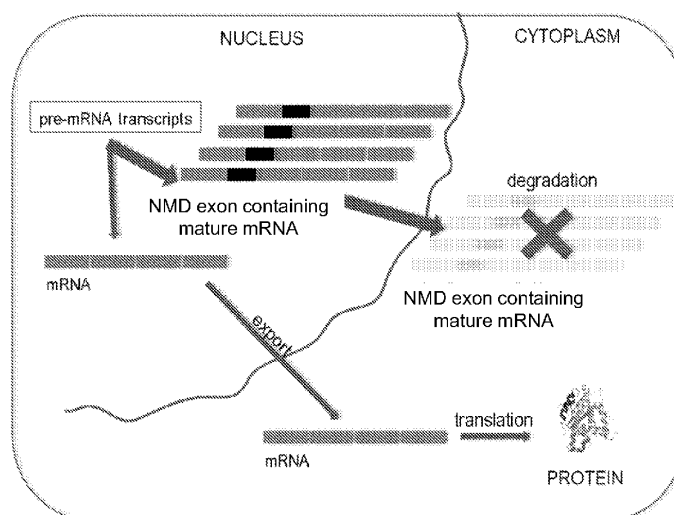


FIG. 1A

(57) Abstract: Alternative splicing events in genes can lead to non-productive mRNA transcripts which in turn can lead to aberrant protein expression, and therapeutic agents which can target the alternative splicing events in genes can modulate the expression level of functional proteins in patients and/or inhibit aberrant protein expression. Such therapeutic agents can be used to treat a condition or disease caused by protein deficiency and/or mitochondrial function deficit.

[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))*