



(51) International Patent Classification:

C12N 15/113 (2010.01) A61K 31/7125 (2006.01)
A61K 31/712 (2006.01) A61P 25/08 (2006.01)

(21) International Application Number:

PCT/US2024/039369

(22) International Filing Date:

24 July 2024 (24.07.2024)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

63/515,284 24 July 2023 (24.07.2023) US
63/569,003 22 March 2024 (22.03.2024) US

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

(72) Inventors: **TICHO, Baruch**; c/o Stoke Therapeutics, Inc.,
45 Wiggins Avenue, Bedford, Massachusetts 01730 (US).
PARKERSON, Kimberly; c/o Stoke Therapeutics, Inc.,
45 Wiggins Avenue, Bedford, Massachusetts 01730 (US).
MEENA, Meena; c/o Stoke Therapeutics, Inc., 45 Wiggins
Avenue, Bedford, Massachusetts 01730 (US). **MOHAPA-**

TRA, Susovan; c/o Stoke Therapeutics, Inc., 45 Wiggins
Avenue, Bedford, Massachusetts 01730 (US).

(74) Agent: **NIP, Lisa** et al.; Wilson Sonsini Goodrich & Rosati,
650 Page Mill Road, Palo Alto, California 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, 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: METHODS FOR TREATING CONDITIONS AND DISEASES

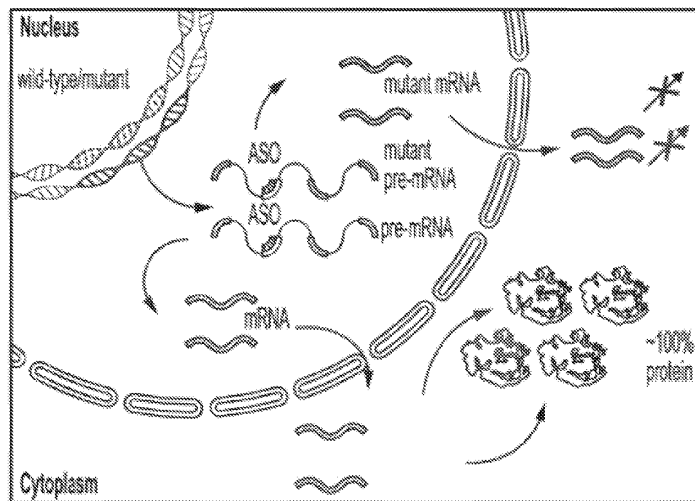


FIG. 1B

(57) Abstract: Provided herein are methods for treating conditions and disease characterized by SCN1A, SCN8A or SCN5A protein deficiency by targeting the alternative splicing events in the *SCN1A* gene and modulating the expression level of functional proteins in Dravet Syndrome patients and/or inhibit aberrant protein expression.

[Continued on next page]

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:

- *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))*