

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
11 August 2022 (11.08.2022)



(10) International Publication Number
WO 2022/169947 A3

- (51) **International Patent Classification:**
C12N 15/113 (2006.01) *A61K 9/00* (2006.01)
- (21) **International Application Number:**
PCT/US2022/015074
- (22) **International Filing Date:**
03 February 2022 (03.02.2022)
- (25) **Filing Language:** English
- (26) **Publication Language:** English
- (30) **Priority Data:**
63/145,288 03 February 2021 (03.02.2021) US
- (71) **Applicant:** **STOKE THERAPEUTICS, INC.** [US/US];
45 Wiggins Ave., Bedford, MA 01730 (US).
- (72) **Inventors:** **AZNAREZ, Isabel**; c/o Stoke Therapeutics,
Inc., 45 Wiggins Ave., Bedford, MA 01730 (US). **KACH,
Jacob, Albert**; c/o Stoke Therapeutics, Inc., 45 Wiggins
Ave., 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,
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))

(54) **Title:** COMPOSITIONS FOR TREATMENT OF CONDITIONS AND DISEASES ASSOCIATED WITH POLYCYSTIN EXPRESSION

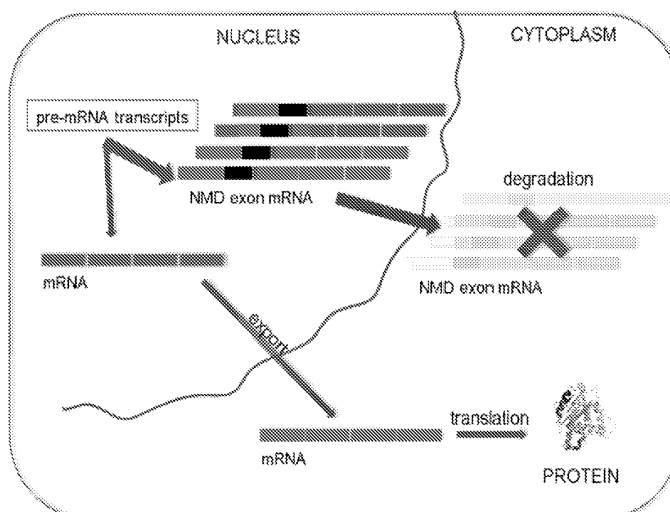


FIG. 1A

(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.

[Continued on next page]

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))*

(88) Date of publication of the international search report:
15 September 2022 (15.09.2022)