

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
12 November 2020 (12.11.2020)



(10) International Publication Number
WO 2020/227097 A1

(51) International Patent Classification:

A61K 31/4192 (2006.01) C07C 13/465 (2006.01)
A61K 31/015 (2006.01) C07D 249/04 (2006.01)
A61K 31/16 (2006.01)

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).

(21) International Application Number:

PCT/US2020/031039

(22) International Filing Date:

01 May 2020 (01.05.2020)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

62/842,855	03 May 2019 (03.05.2019)	US
62/842,858	03 May 2019 (03.05.2019)	US
62/842,861	03 May 2019 (03.05.2019)	US
62/982,858	28 February 2020 (28.02.2020)	US

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))

(71) Applicant: PRAXIS PRECISION MEDICINES, INC.

[US/US]; One Broadway, 16th Floor, Cambridge, MA 02142 (US).

(72) Inventors: MARTINEZ BOTELLA, Gabriel; 17 Par-

menter Road, Wayland, MA 01778 (US). GRIFFIN, Andrew, Mark; 232 Rue Barette, L'Ile Bizard, Quebec H9C2Y (CA). CHARIFSON, Paul, S.; 7 Dartmouth Drive, Framingham, MA 01701 (US). REDDY, Kiran; 14 9th Street, Unit 14, Boston, MA 02129 (US). KAHLIG, Michael, Kristopher Mathieu; 1130 James Avenue, Redwood City, CA 94062 (US). MARRON, Brian, Edward; 1955 Augusta Valley Ln SE, Ada, MI 49301 (US).

(74) Agent: CHOI, Boyeon et al.; Goodwin Procter LLP, 100

Northern Avenue, IP Docketing Dept./7th Fl, Boston, MA 02210 (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, 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,

(54) Title: KCNT1 INHIBITORS AND METHODS OF USE

(57) Abstract: The present invention is directed to, in part, compounds and compositions useful for preventing and/or treating a neurological disease or disorder, a disease or condition relating to excessive neuronal excitability, and/or a gain-of-function mutation in a gene (e.g., KCNT1). Methods of treating a neurological disease or disorder, a disease or condition relating to excessive neuronal excitability, and/or a gain-of-function mutation in a gene such as KCNT1 are also provided herein.



WO 2020/227097 A1