

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
02 November 2023 (02.11.2023)



(10) International Publication Number
WO 2023/211852 A1

(51) International Patent Classification:

A61K 31/44 (2006.01) A61K 31/424 (2006.01)
A61K 31/4192 (2006.01) A61K 31/33 (2006.01)

(21) International Application Number:

PCT/US2023/019652

(22) International Filing Date:

24 April 2023 (24.04.2023)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

63/335,204	26 April 2022 (26.04.2022)	US
63/349,402	06 June 2022 (06.06.2022)	US
63/357,944	01 July 2022 (01.07.2022)	US

(71) Applicant: PRAXIS PRECISION MEDICINES, INC.

[US/US]; 99 High Street, 30th Floor, Boston, MA 02110 (US).

(72) Inventors; and

(71) Applicants: KAHLIG, Kristopher, Mathieu [US/US]; c/o Praxis Precision Medicines, 99 High Street, 30th Floor, Boston, MA 02110 (US). WITTMANN, Marion [DE/US]; c/o Praxis Precision Medicines, 99 High Street, 30th Floor, Boston, MA 02110 (US). HUGHES, Zoe, A. [US/US]; c/o Praxis Precision Medicines, 99 High Street, 30th Floor, Boston, MA 02110 (US). RAVINA, Bernard [US/US]; c/o Praxis Precision Medicines, 99 High Street, 30th Floor, Boston, MA 02110 (US).

(74) Agent: MARGOLIN, Yelena et al.; McCarter & English, LLP, 265 Franklin Street, Boston, MA 02110 (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,

(54) Title: TREATMENT OF NEUROLOGICAL DISORDERS

(57) Abstract: The present disclosure is generally directed to methods of treating a disease, disorder, or condition, e.g., a neurological disorder, a disorder associated with excessive neuronal excitability, or a disorder associated with *de novo* gain-of-function or loss-of-function mutations in major central nervous system sodium channel genes, such as for example, *SCN1A*, *SCN2A*, and *SCN8A*, using a compound of Formula (I) as defined herein or a pharmaceutically acceptable salt thereof.

WO 2023/211852 A1