

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
08 October 2020 (08.10.2020)



(10) International Publication Number
WO 2020/206080 A1

(51) International Patent Classification:

A61P 11/00 (2006.01) C07D 515/08 (2006.01)
C07D 239/69 (2006.01)

(21) International Application Number:

PCT/US2020/026331

(22) International Filing Date:

02 April 2020 (02.04.2020)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

62/828,699 03 April 2019 (03.04.2019) US

(71) Applicant: VERTEX PHARMACEUTICALS INCORPORATED [US/US]; 50 Northern Avenue, Boston, Massachusetts 02210 (US).

(72) Inventors: ABELA, Alexander Russell; 50 Northern Avenue, Boston, Massachusetts 02210 (US). ABRAHAM, Sunny; 50 Northern Avenue, Boston, Massachusetts 02210 (US). ANDERSON, Corey Don; 50 Northern Avenue, Boston, Massachusetts 02210 (US). ARUMUGAM, Vijayalaksmi; 50 Northern Avenue, Boston, Massachusetts 02210 (US). CHAU, Jaclyn; 50 Northern Avenue, Boston, Massachusetts 02210 (US). CLEMENS, Jeremy; 50 Northern Avenue, Boston, Massachusetts 02210 (US). CLEVELAND, Thomas; 50 Northern Avenue, Boston, Massachusetts 02210 (US). DWIGHT, Timothy A.; 50 Northern Avenue, Boston, Massachusetts 02210 (US). FRIEMAN, Bryan A.; 50 Northern Avenue, Boston, Massachusetts 02210 (US).

(72) Inventor: GROOTENHUIS, Peter (deceased) (deceased).

(72) Inventors: HADIDA RUAH, Sara Sabina; 50 Northern Avenue, Boston, Massachusetts 02210 (US). ISHIIHARA, Yoshihiro; 50 Northern Avenue, Boston, Massachusetts 02210 (US). KRENITSKY, Paul; 50 Northern Avenue, Boston, Massachusetts 02210 (US). MCCARTNEY, Jason; 50 Northern Avenue, Boston, Massachusetts 02210 (US).

(US). MELILLO, Vito; 50 Northern Avenue, Boston, Massachusetts 02210 (US). MILLER, Mark Thomas; 50 Northern Avenue, Boston, Massachusetts 02210 (US). SILINA, Alina; 50 Northern Avenue, Boston, Massachusetts 02210 (US). UY, Johnny; 50 Northern Avenue, Boston, Massachusetts 02210 (US). ZHOU, Jinglan; 50 Northern Avenue, Boston, Massachusetts 02210 (US).

(74) Agent: MCDONELL, Leslie A. et al.; Finnegan, Henderson, Farabow, Garrett & Dunner, LLP, 901 New York Avenue, NW, Washington, District of Columbia 20001 (US).

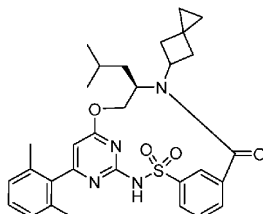
(81) Designated States (unless otherwise indicated, for every kind of national protection available): AE, AG, AL, AM, AO, AT, AU, AZ, BA, BB, BG, BH, BN, BR, BW, BY, BZ, CA, CH, CL, CN, CO, CR, CU, CZ, DE, DJ, DK, DM, DO, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, GT, HN, HR, HU, ID, IL, IN, IR, IS, 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, 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).

Published:

— with international search report (Art. 21(3))

(54) Title: CYSTIC FIBROSIS TRANSMEMBRANE CONDUCTANCE REGULATOR MODULATING AGENTS



(I)

(57) Abstract: Compound (I), deuterated derivatives, and pharmaceutically acceptable salts of any of the foregoing are disclosed. Methods of treating cystic fibrosis using these compounds are also disclosed.



WO 2020/206080 A1