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

(19) World Intellectual Property Organization
International Bureau

(43) International Publication Date
24 March 2005 (24.03.2005)

PCT

(10) International Publication Number
WO 2005/026137 A2

(51) International Patent Classification[7]: C07D 277/82, 277/46, 417/12, 263/48, 417/04, 417/06, A61K 31/426, A61P 43/00

(21) International Application Number:
PCT/US2004/029206

(22) International Filing Date:
7 September 2004 (07.09.2004)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
60/500,444 6 September 2003 (06.09.2003) US

(71) Applicants *(for all designated States except US)*: **VERTEX PHARMACEUTICALS INCORPORATED** [US/US]; 130 Waverly Street, Cambridge, MA 02139-4242 (US). **CLEVELAND, Thomas** [US/US]; 4431 Vision Drive #7, San Diego, CA 92121 (US). **MAKINGS, Lewis R.** [US/US]; 337 Cerro Street, Encinitas, CA 92024 (US). **HAMILTON, Matthew** [US/US]; 3005 Ridgecliff Lane, Spring Valley, CA 91977 (US). **GROOTENHUIS, Peter D.J.** [NL/US]; 4801 Riding Ridge Road, San Diego, CA 92122 (US).

(72) Inventors; and

(75) Inventors/Applicants *(for US only)*: **MILLER, Mark T.** [US/US]; 5075 La Jolla Blvd., San Diego, CA 92109 (US). **HADIDA RUAH, Sara S.** [ES/US]; 3717 Nobel Drive #1401, San Diego, CA 92122 (US). **SINGH, Ashavini K.** [IN/US]; 13597 Tiverton Road, San Diego, CA 92130 (US).

(74) Agents: **HALEY, James F., Jr.** et al.; c/o Fish & Neave, 1251 Avenue of the Americas, New York, NY 10020 (US).

(81) Designated States *(unless otherwise indicated, for every kind of national protection available)*: AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BW, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NA, NI, NO, NZ, OM, PG, PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, SY, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, YU, ZA, ZM, ZW.

(84) Designated States *(unless otherwise indicated, for every kind of regional protection available)*: ARIPO (BW, GH, GM, KE, LS, MW, MZ, NA, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IT, LU, MC, NL, PL, PT, RO, SE, SI, SK, TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).

Published:
— *without international search report and to be republished upon receipt of that report*

For two-letter codes and other abbreviations, refer to the "Guidance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette.

WO 2005/026137 A2

(54) Title: MODULATORS OF ATP-BINDING CASSETTE TRANSPORTERS

(57) Abstract: The present invention relates to modulators of ATP-Binding Cassette ("ABC") transporters or fragments thereof, including CF Transmembrane Regulator ("CFTR"), compositions thereof, and methods therewith. The present invention also relates to methods of treating ABC transporter mediated diseases using such modulators.