



(51) International Patent Classification:

C07D 471/04 (2006.01) A61P 13/12 (2006.01)
C07D 519/00 (2006.01) A61K 31/4355 (2006.01)

(21) International Application Number:

PCT/US2024/053437

(22) International Filing Date:

29 October 2024 (29.10.2024)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

63/594,357 30 October 2023 (30.10.2023) US

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

(72) Inventors: AUSTIN, James Robert; 50 Northern Avenue, Boston, Massachusetts 02210 (US). BOYALL, Dean; 50 Northern Avenue, Boston, Massachusetts 02210 (US). CHUDYK, Ewa Iwona; 50 Northern Avenue, Boston, Massachusetts 02210 (US). DENEKA, Dawid; 50 Northern Avenue, Boston, Massachusetts 02210 (US). DODD, James; 50 Northern Avenue, Boston, Massachusetts 02210 (US). EVERITT, Simon Robert; 50 Northern Avenue, Boston, Massachusetts 02210 (US). FAUST, Belinda Rafaella; 50 Northern Avenue, Boston, Massachusetts 02210 (US). FRAYSSE, Damien Jacques Michel; 50 Northern Avenue, Boston, Massachusetts 02210 (US). GLADDING, Jeffery Allen; 50 Northern Avenue, Boston, Massachusetts 02210 (US). HEPBURN, Hamish Bruce; 50 Northern Avenue, Boston, Massachusetts 02210 (US). KEILY, Shazia; 50 Northern Avenue, Boston, Massachusetts 02210 (US). KNEGTEL, Ronald Marcellus; 50 Northern Avenue, Boston, Massachusetts 02210 (US). MAXWELL, Brad D.; 50 Northern Avenue, Boston, Massachusetts 02210 (US). MUI, James Jun Bon; 50 Northern Avenue, Boston, Massachusetts 02210 (US). NASRALLAH, Chady; 50 Northern Avenue, Boston, Massachusetts 02210 (US). PINDER, Joanne Louise; 50 Northern Avenue, Boston, Massachusetts 02210 (US).

RUTHERFORD, Alistair Peter; 50 Northern Avenue, Boston, Massachusetts 02210 (US). SALMAN, Alan Alessandro; 50 Northern Avenue, Boston, Massachusetts 02210 (US). WRIGHT, Miranda; 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, 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, 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, ME, 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: COMPOUNDS FOR THE TREATMENT OF KIDNEY DISEASE

(57) Abstract: The disclosure provides polycystin-1 (PC1) correctors, pharmaceutical compositions containing at least one such corrector, methods of treating autosomal dominant polycystic kidney disease using such correctors and pharmaceutical compositions, and processes for making such correctors.

