

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
14 December 2023 (14.12.2023)



(10) International Publication Number
WO 2023/240267 A3

(51) International Patent Classification:

C07D 405/12 (2006.01) C07D 491/056 (2006.01)
C07D 405/14 (2006.01) C07D 519/00 (2006.01)
C07D 413/12 (2006.01) A61P 11/00 (2006.01)
C07D 471/04 (2006.01)

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))
- 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))

(21) International Application Number:

PCT/US2023/068239

(22) International Filing Date:

09 June 2023 (09.06.2023)

(88) Date of publication of the international search report:

18 January 2024 (18.01.2024)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

63/350,970 10 June 2022 (10.06.2022) US

(71) Applicant: SIONNA THERAPEUTICS [US/US]; 22
Strathmore Road, Natick, MA 01760 (US).

(72) Inventors: LIAO, Junkai; 33 Noel Road, Andover, MA
01810 (US). MUNSON, Mark; 45 Central Street, Ac-
ton, MA 01720 (US). GAO, Zhongli; 40 Myrtle Street,
Unit 11, Waltham, MA 02453 (US). HURLBUT, Gregory;
140 Waite Road, Boxborough, MA 01719 (US). MACOR,
John, E.; 55 Commanders Drive, Washington Crossing, PA
19877 (US).

(74) Agent: HALEY, Christopher, K. et al.; Goodwin Procter
LLP, 100 Northern Avenue, 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, 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,

(54) Title: COMPOUNDS, COMPOSITIONS, AND METHODS OF USING THEREOF

(57) Abstract: The present disclosure relates to heterocyclic compounds, pharmaceutically acceptable salts thereof, and pharmaceutical preparations thereof. Also described herein are compositions and the use of such compounds in methods of treating diseases and conditions mediated by deficient CFTR activity, in particular cystic fibrosis.

WO 2023/240267 A3