

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
02 January 2025 (02.01.2025)



(10) International Publication Number
WO 2025/006680 A1

(51) International Patent Classification:
A61K 31/519 (2006.01) *A61P 7/00* (2006.01)
A61K 45/06 (2006.01)

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).

(21) International Application Number:
PCT/US2024/035719

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))

(22) International Filing Date:
27 June 2024 (27.06.2024)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
63/524,530 30 June 2023 (30.06.2023) US
63/535,183 29 August 2023 (29.08.2023) US
63/607,818 08 December 2023 (08.12.2023) US
63/637,286 22 April 2024 (22.04.2024) US

(71) Applicant: **PRINCIPIA BIOPHARMA INC.** [US/US];
55 Corporate Drive, Bridgewater, New Jersey 08807 (US).

(72) Inventors: **DAAK, Ahmed**; c/o Sanofi, Patent Department,
450 Water Street, Cambridge, Massachusetts 02141 (US).
LEE, Michelle; c/o Sanofi, Patent Department, 450 Water
Street, Cambridge, Massachusetts 02141 (US). **GUO, Hail-
ing**; c/o Sanofi, Patent Department, 450 Water Street, Cam-
bridge, Massachusetts 02141 (US).

(74) Agent: **WINKLER, Lauren** et al.; 125 Cambridge Park
Drive, Suite 301, Cambridge, Massachusetts 02140 (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,

(54) Title: METHODS FOR TREATING PERSISTENT OR CHRONIC IMMUNE THROMBOCYTOPENIA
IN CHILDREN, ADOLESCENTS AND ADULTS BY ADMINISTERING (R)-2-[3-[4-AMINO-3-(2-FLUO-
RO-4-PHENOXY-PHENYL)PYRAZOLO[3,4-D]PYRIMIDIN-L-YL]PIPERIDINE-L-CARBONYL]-4-METHYL-4-[4-(OXE-
TAN-3-YL)PIPERAZIN-L-YL]PENT-2-ENENITRILE

(57) Abstract: Methods for treating children, adolescents and adults with persistent or chronic immune thrombocytopenia com-
prising administering at least one compound chosen from (R)-2-[3-[4-amino-3-(2-fluoro-4-phenoxy-phenyl)pyrazolo[3,4-d]pyrim-
idin-1-yl]piperidine-1-carbonyl]-4-methyl-4-[4-(oxetan-3-yl)piperazin-1-yl]pent-2-enenitrile (rilzabrutinib) and pharmaceutically ac-
ceptable salts thereof are disclosed.

WO 2025/006680 A1