

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
06 March 2025 (06.03.2025)



(10) International Publication Number
WO 2025/049558 A1

(51) International Patent Classification:
C12N 15/113 (2010.01) C12Q 1/6883 (2018.01)
A61K 31/713 (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/US2024/044160

Declarations under Rule 4.17:

- as to applicant's entitlement to apply for and be granted a patent (Rule 4.17(ii))
- as to the applicant's entitlement to claim the priority of the earlier application (Rule 4.17(iii))

(22) International Filing Date:
28 August 2024 (28.08.2024)

(25) Filing Language: English

(26) Publication Language: English

Published:

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

(30) Priority Data:
63/535,082 29 August 2023 (29.08.2023) US
63/547,633 07 November 2023 (07.11.2023) US

(71) Applicant: **REGENERON PHARMACEUTICALS, INC.** [US/US]; 777 Old Saw Mill River Road, Tarrytown, New York 10591 (US).

(72) Inventors: **LOTTA, Luca Andrea**; 777 Old Saw Mill River Road, Tarrytown, New York 10591 (US). **HINDY, George**; 777 Old Saw Mill River Road, Tarrytown, New York 10591 (US). **GUSAROVA, Viktoria**; 777 Old Saw Mill River Road, Tarrytown, New York 10591 (US). **SLEEMAN, Mark**; 777 Old Saw Mill River Road, Tarrytown, New York 10591 (US). **ADAM, Rene Christian**; 777 Old Saw Mill River Road, Tarrytown, New York 10591 (US).

(74) Agent: **LEGAARD, Paul K.** et al.; Armstrong Teasdale LLP, 7700 Forsyth Boulevard, Suite 1800, St. Louis, Missouri 63105 (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: TREATMENT OF LIVER DISEASE OR METABOLIC DISORDER WITH FOLLICULIN INTERACTING PROTEIN 1 (FNIP1) INHIBITORS AND/OR FOLLICULIN (FLCN) INHIBITORS

(57) Abstract: The present disclosure generally relates to the treatment of subjects having liver disease or metabolic disorder or at risk of developing liver disease or metabolic disorder by administering a Folliculin Interacting Protein 1 (FNIP1) inhibitor and/or a Folliculin (FLCN) inhibitor to the subject.

WO 2025/049558 A1