

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
23 January 2025 (23.01.2025)



(10) International Publication Number
WO 2025/019486 A2

(51) International Patent Classification:
A61K 45/06 (2006.01)

(21) International Application Number:
PCT/US2024/038183

(22) International Filing Date:
16 July 2024 (16.07.2024)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
63/527,123 17 July 2023 (17.07.2023) US

(71) Applicant: **Q32 BIO INC.** [US/US]; 830 Winter Street,
Waltham, MA 02451 (US).

(72) Inventors: **WAWERSIK, Stefan**; c/o Q32 Bio Inc., 830
Winter Street, Waltham, MA 02451 (US). **VIOLETTE,
Shelia Marie**; c/o Q32 Bio Inc., 830 Winter Street,
Waltham, MA 02451 (US). **MORGAN, Jennifer**; c/o
Q32 Bio Inc., 830 Winter Street, Waltham, MA 02451
(US). **RYAN, Sarah**; c/o Q32 Bio Inc., 830 Winter Street,
Waltham, MA 02451 (US). **GALAND, Claire**; c/o Q32 Bio
Inc., 830 Winter Street, Waltham, MA 02451 (US). **THUR-
MAN, Joshua M.**; c/o Q32 Bio Inc., 830 Winter Street,
Waltham, MA 02451 (US).

(74) Agent: **LU, Yu** et al.; McCarter & English, LLP, 265
Franklin Street, Boston, MA 02110 (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).

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))

Published:

- without international search report and to be republished upon receipt of that report (Rule 48.2(g))
- with sequence listing part of description (Rule 5.2(a))

(54) Title: TARGETED TREATMENT OF COMPLEMENT-MEDIATED DISEASE THROUGH LOCAL COMPLEMENT INHIBITION BASED ON URINARY UC5b-9 DETECTION

(57) Abstract: Provided is the use of urinary C5b-9 (uC5b-9) as a highly accurate and superior biomarker well correlated with complement activity in local tissues affected by complement-mediated diseases (e.g., those with a kidney damage component). The biomarker is useful, for example, in treating and/or monitoring such complement-mediated diseases, using complement inhibitors targeted to such local tissues affected by the diseases, without systemic complement inhibition.



WO 2025/019486 A2