

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

(19) World Intellectual Property
Organization

International Bureau

(43) International Publication Date
22 September 2022 (22.09.2022)



(10) International Publication Number
WO 2022/195074 A3

(51) International Patent Classification:

A61K 48/00 (2006.01) C12N 15/86 (2006.01)

(21) International Application Number:

PCT/EP2022/057145

(22) International Filing Date:

18 March 2022 (18.03.2022)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

63/163,393 19 March 2021 (19.03.2021) US

(71) Applicant: **UCL BUSINESS LTD** [GB/GB]; 97 Tottenham Court Road, The Network Building, London W1T 4 TP (GB).

(72) Inventors: **VOIT, Thomas**; c/o UCL BUSINESS LTD, 97 Tottenham Court Road, The Network Building, London W1T 4 TP (GB). **DUMONCEAUX, Julie**; c/o UCL BUSINESS LTD, 97 Tottenham Court Road, The Network Building, London W1T 4 TP (GB). **ELLIOTT, Perry**; c/o UCL BUSINESS LTD, 97 Tottenham Court Road, The Network Building, London W1T 4 TP (GB). **MARIOT, Virginie**; c/o UCL BUSINESS LTD, 97 Tottenham Court Road, The Network Building, London W1T 4 TP (GB).

(74) Agent: **MAIWALD GMBH**; Dr. Andrea Lasar, Elisenhof, Eisenstrasse 3, 80335 Munich (DE).

(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, CZ, DE, DJ, DK, DM, DO, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, GT, HN, HR, HU, ID, IL, IN, IR, IS, IT, JM, JO, JP, KE, KG, KH, KN, KP, KR, KW, KZ, LA, LC, LK, LR, LS, LU, LY, MA, MD, ME, MG, MK, MN, 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, GH, GM, KE, LR, LS, MW, MZ, NA, RW, 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, 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))
- 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))
- with sequence listing part of description (Rule 5.2(a))
- in black and white; the international application as filed contained color or greyscale and is available for download from PATENTSCOPE

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

10 November 2022 (10.11.2022)

(54) Title: GENE THERAPY COMPOSITION AND TREATMENT OF RIGHT VENTRICULAR ARRHYTHMOGENIC CARDIOMYOPATHY

(57) Abstract: Disclosed are a composition and method of treating or preventing cardiomyopathy in a human subject. In some embodiments, the method comprises delivering a therapeutic dose of a gene therapy vector to cardiomyocytes of the human subject, wherein the gene therapy vector comprises a nucleic acid sequence encoding for PKP2.

WO 2022/195074 A3