

(19) World Intellectual Property
Organization
International Bureau



(10) International Publication Number

WO 2023/220657 A9

(43) International Publication Date
16 November 2023 (16.11.2023)

WIPO | PCT

- (51) **International Patent Classification:**
G01N 33/68 (2006.01) G01N 33/92 (2006.01) 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/US2023/066852
- (22) **International Filing Date:**
10 May 2023 (10.05.2023)
- (25) **Filing Language:** English
- (26) **Publication Language:** English
- (30) **Priority Data:**
63/340,412 10 May 2022 (10.05.2022) US
63/351,380 11 June 2022 (11.06.2022) US
- (71) **Applicant:** LARIMAR THERAPEUTICS, INC.
[US/US]; Three Bala Plaza East, Suite 506, Bala Cynwyd, PA 19004 (US).
- (72) **Inventors:** BETTOUN, Joan, David; 8106 Cadwalader Avenue, Elkins Park, PA 19027 (US). ROSIERS, Christine, Des; 19 Chemin Du Hudson Club, Rigaud, QC J0P 1P0 (CA). FOREST, Anik; 2027 Montee Du Cheval Aile, Sainte-Lucie-des-Laurentides, QC J0T 2J0 (CA).
- (74) **Agent:** MELLO, Jill, Ann 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,

(54) **Title:** FRATAXIN-SENSITIVE LIPID MARKERS FOR MONITORING FRATAXIN REPLACEMENT THERAPY

(57) **Abstract:** The present disclosure is based, at least in part, on providing a set of markers, also referred to herein as FXN-sensitive lipid markers (or FSLMs), the respective levels of which are positively or negatively correlated to frataxin (FXN) levels in a cell. Therefore, these FSLMs can be used to determine, evaluate, and/or monitor the effectiveness of FXN replacement therapy in a subject.

WO 2023/220657 A9