

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
09 September 2022 (09.09.2022)



(10) International Publication Number
WO 2022/187463 A1

- (51) **International Patent Classification:**
A61K 31/4468 (2006.01) *A61P 3/06* (2006.01)
- (21) **International Application Number:**
PCT/US2022/018672
- (22) **International Filing Date:**
03 March 2022 (03.03.2022)
- (25) **Filing Language:** English
- (26) **Publication Language:** English
- (30) **Priority Data:**
63/155,960 03 March 2021 (03.03.2021) US
63/165,457 24 March 2021 (24.03.2021) US
- (71) **Applicant: AMRYT PHARMACEUTICALS INC.**
[US/US]; 160 Federal Street, 21st Floor, Boston, Massachusetts 02110 (US).

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).

(72) **Inventor: SUMERAY, Mark;** c/o Amryt Pharmaceuticals Inc., 160 Federal Street, 21st Floor, Boston, Massachusetts 02110 (US).

Declarations under Rule 4.17:

— as to the applicant's entitlement to claim the priority of the earlier application (Rule 4.17(iii))

(74) **Agent: BROWN, Fraser et al.;** Cooley LLP, 1299 Pennsylvania Ave., Suite 700, Washington, District of Columbia 20004 (US).

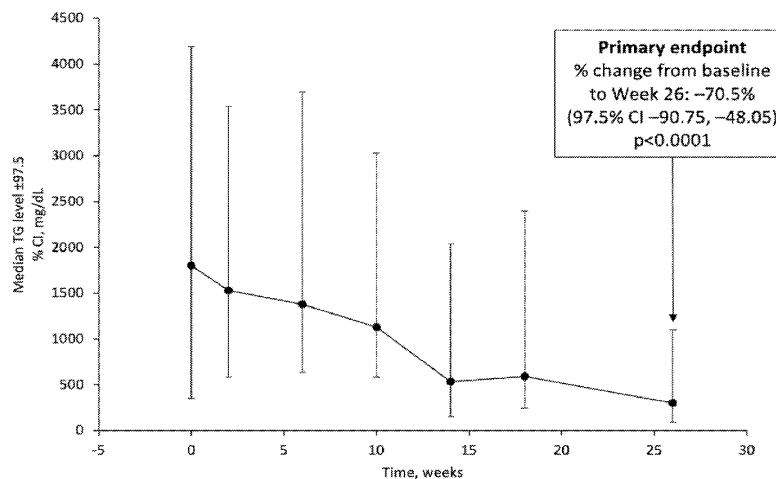
Published:

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

(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,

(54) **Title:** METHODS FOR TREATING FAMILIAL CHYLOMICRONEMIA SYNDROME

FIG. 7



(57) **Abstract:** Provided herein are methods of treating familial chylomicronemia syndrome (FCS) with compositions comprising lomitapide or a pharmaceutically acceptable salt thereof.

WO 2022/187463 A1