

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

(19) World Intellectual Property
Organization
International Bureau



(10) International Publication Number
WO 2022/187435 A1

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

(51) International Patent Classification:
C12N 15/113 (2010.01) A61P 3/06 (2006.01)
A61K 31/713 (2006.01)

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).

(21) International Application Number:
PCT/US2022/018630

Published:

(22) International Filing Date:
03 March 2022 (03.03.2022)

- 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))

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
63/156,476 04 March 2021 (04.03.2021) US
63/308,668 10 February 2022 (10.02.2022) US

(71) Applicant: ALNYLAM PHARMACEUTICALS, INC.
[US/US]; 675 West Kendall Street, Henri A. Termeer
Square, Cambridge, MA 02142 (US).

(72) Inventors: BONDURANT, Lucas, D.; 675 West Kendall
Street, Henri A. Termeer Square, Cambridge, MA 02142
(US). SCHLEGEL, Mark, K.; 675 West Kendall Street,
Henri A. Termeer Square, Cambridge, MA 02142 (US).
ZUBER, Jeffrey; 675 West Kendall Street, Henri A. Ter-
meer Square, Cambridge, MA 02142 (US). WOODS, Lau-
ren, Blair; 675 West Kendall Street, Henri A. Termeer
Square, Cambridge, MA 02142 (US). CHICKERING,
Tyler; 675 West Kendall Street, Henri A. Termeer Square,
Cambridge, MA 02142 (US).

(74) Agent: ZACHARAKIS, Maria, Laccotripe et al.; Mc-
Carter & 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, 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,

(54) Title: ANGIOPOIETIN-LIKE 3 (ANGPTL3) IRNA COMPOSITIONS AND METHODS OF USE THEREOF

(57) Abstract: The present invention relates to RNAi agents, e.g., double stranded RNA (dsRNA) agents, targeting the Angiopoietin-like 3 (ANGPTL3) gene. The invention also relates to methods of using such RNAi agents to inhibit expression of an ANGPTL3 gene and to methods of preventing and treating an ANGPTL3-associated disorder, e.g., a disorder of lipid metabolism, such as hyperlipidemia or hypertriglyceridemia.

WO 2022/187435 A1