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

(19) World Intellectual Property Organization
International Bureau

(43) International Publication Date
14 October 2021 (14.10.2021)

WIPO | PCT

(10) International Publication Number
WO 2021/207189 A1

(51) International Patent Classification:
C12N 15/113 (2010.01)

(21) International Application Number:
PCT/US2021/025956

(22) International Filing Date:
06 April 2021 (06.04.2021)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
63/006,328 07 April 2020 (07.04.2020) US
63/161,313 15 March 2021 (15.03.2021) US

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

(72) Inventors: **CANTLEY, William**; 675 West Kendall Street, Henri A. Termeer Square, Cambridge, MA 02142 (US). **MCININCH, James, D.**; 675 West Kendall Street, Henri A. Termeer Square, Cambridge, MA 02142 (US). **CASTORENO, Adam**; 675 West Kendall Street, Henri A. Termeer Square, Cambridge, MA 02142 (US). **KAITTANIS, Charalambos**; 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).

(74) Agent: **LU, David** et al.; Lando & Anastasi, LLP, 60 State Street, 23rd Floor, Boston, MA 02109 (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, 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).

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:
— *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))*

(54) Title: COMPOSITIONS AND METHODS FOR SILENCING SCN9A EXPRESSION

(57) Abstract: The disclosure relates to double-stranded ribonucleic acid (dsRNA) compositions targeting SCN9A, and methods of using such dsRNA compositions to alter (e.g., inhibit) expression of SCN9A.

WO 2021/207189 A1