

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

(19) World Intellectual Property Organization
International Bureau



(43) International Publication Date
3 June 2011 (03.06.2011)

(10) International Publication Number
WO 2011/066475 A1

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

(21) International Application Number:
PCT/US2010/058123

(22) International Filing Date:
25 November 2010 (25.11.2010)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
61/264,668 26 November 2009 (26.11.2009) US
61/295,721 17 January 2010 (17.01.2010) US
61/372,072 9 August 2010 (09.08.2010) US

(71) Applicant (for all designated States except US): **QUARK PHARMACEUTICALS, INC.** [US/US]; 6501 Dumbarton Circle, Fremont, California 94555 (US).

(72) Inventor; and

(75) Inventor/Applicant (for US only): **AVKIN-NACHUM, Sharon** [IL/IL]; 4 Hakramim Street, 74054 Nes Zionna (IL).

(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, BR, BW, BY, BZ,

CA, CH, CL, CN, CO, CR, CU, CZ, DE, DK, DM, DO, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, GT, HN, HR, HU, ID, IL, IN, IS, JP, KE, KG, KM, KN, KP, KR, KZ, LA, LC, LK, LR, LS, LT, LU, LY, MA, MD, ME, MG, MK, MN, MW, MX, MY, MZ, NA, NG, NI, NO, NZ, OM, PE, PG, PH, PL, PT, RO, RS, RU, SC, SD, SE, SG, SK, SL, SM, ST, SV, SY, TH, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, 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, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian (AM, AZ, BY, KG, KZ, MD, 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, 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))

(54) Title: SIRNA COMPOUNDS COMPRISING TERMINAL SUBSTITUTIONS

(57) Abstract: The invention relates to modified siRNA compounds which down-regulate target gene expression, to pharmaceutical compositions comprising such compounds and to methods of treating and/or preventing the incidence or severity of various diseases or conditions associated with the genes and/or symptoms associated with such diseases or conditions.



WO 2011/066475 A1