

(19) World Intellectual Property Organization
International Bureau



(43) International Publication Date
21 February 2008 (21.02.2008)

PCT

(10) International Publication Number
WO 2008/022309 A2

(51) International Patent Classification:
C12Q 1/68 (2006.01)

(21) International Application Number:
PCT/US2007/076202

(22) International Filing Date: 17 August 2007 (17.08.2007)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
60/822,833 18 August 2006 (18.08.2006) US
60/915,868 3 May 2007 (03.05.2007) US
11/840,468 17 August 2007 (17.08.2007) US

(71) Applicant (for all designated States except US): **MIRUS BIO CORPORATION** [US/US]; 505 S. Rosa Rd., Madison, WI 53719 (US).

(72) Inventors; and

(75) Inventors/Applicants (for US only): **ROZEMA, David, B.** [US/US]; 9418 Whippoorwill Way, Middleton, WI 53562 (US). **WAKEFIELD, Darren, H.** [US/US]; 2790 Lyman Lane, Fitchburg, WI 53711 (US). **WOLFF, Jon, A.** [US/US]; 1122 University Bay Drive, Madison, WI 53705 (US). **WONG, So** [US/US]; 435 Landover Dr., Oregon, WI 53575 (US). **KLEIN, Jason** [US/US]; 1462 Bellflower Ln., Madison, WI 53713 (US). **LEWIS, David, L.** [US/US]; 505 Maple Avenue, Madison, WI

53704 (US). **HAGSTROM, James, E.** [US/US]; 4700 Bergamot Way, Middleton, WI 53562 (US). **BLOKHIN, Andrei** [US/US]; 5770 Dawley Drive, Fitchburg, WI 53711 (US). **TRUBETSKOY, Vladimir** [US/US]; 7435 Hunters Court, Middleton, WI 53562 (US). **HERWEIJER, Hans** [NL/US]; W 1602 Mortensen Road, Brooklyn, WI 53521 (US).

(81) Designated States (unless otherwise indicated, for every kind of national protection available): AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BH, BR, BW, BY, BZ, CA, CH, 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, PG, PH, PL, PT, RO, RS, RU, SC, SD, SE, SG, SK, SL, SM, SV, SY, 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, LS, MW, MZ, NA, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IS, IT, LT, LU, LV, MC, MT, NL, PL, PT, RO, SE, SI, SK, TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).

Published:

— without international search report and to be republished upon receipt of that report

(54) Title: POLYCONJUGATES FOR *IN VIVO* DELIVERY OF POLYNUCLEOTIDES

(57) Abstract: The present invention is directed to compounds, compositions, and methods useful for delivering polynucleotides or other cell-impermeable molecules to mammalian cells. Described are polyconjugates systems that incorporate targeting, anti-opsonization, anti-aggregation, and transfection activities into small biocompatible *in vivo* delivery vehicles. The use of multiple reversible linkages connecting component parts provides for physiologically responsive activity modulation.

WO 2008/022309 A2