(19) World Intellectual Property Organization

International Bureau

(43) International Publication Date 18 November 2010 (18.11.2010)





(10) International Publication Number WO 2010/132879 A3

(51) International Patent Classification:

C07C 225/04 (2006.01) *C08G 61/12* (2006.01) **A61K** 47/34 (2006.01)

A61K 31/7105 (2006.01)

A61P 35/00 (2006.01)

(21) International Application Number:

PCT/US2010/035127

(22) International Filing Date:

17 May 2010 (17.05.2010)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

61/178,611

15 May 2009 (15.05.2009)

US

- (71) Applicant (for all designated States except US): THE JOHNS HOPKINS UNIVERSITY [US/US]; 3400 North Charles Street, Baltimore, Maryland 21218 (US).
- (72) Inventors; and
- (75) Inventors/Applicants (for US only): GREEN, Jordan, J. [US/US]; 11239 Pear Tree Way, Apt #K, Columbia,

Maryland 21044 (US). SUNSHINE, Joel, C. [US/US]; 707 S. President St., Apt. 640, Baltimore, Maryland 21202 (US). BHISE, Nupura, S. [IN/US]; 500 West University Parkway, 6R, Baltimore, Maryland 21210 (US). SHMUELI, Ron, B. [US/US]; 3100 Saint Paul Street, Baltimore, Maryland 21218 (US). TZENG, Stephany, Y. [US/US]; 3501 Saint Paul Street, Apt. 926, Baltimore, Maryland 21218 (US).

- (74) Agent: CHILDERS, Jeffrey, W.; 100 North Charles Street, 5th Floor, Baltimore, Maryland 21201 (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, 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, TT, TZ, UA, UG, US, UZ, VC, VN, ZA, ZM, ZW.

[Continued on next page]

(54) Title: MULTICOMPONENT DEGRADABLE CATIONIC POLYMERS

(57) Abstract: Degradable polymers were synthesized that self-assemble with DNA to form particles that are effective for gene delivery. Small changes to polymer synthesis conditions, particle formulation conditions, and polymer structure provides significant changes to efficacy in a cell-type dependent manner. Polymers presented here are more effective than commercially available materials, such as LIPOFECTAMINE 2000™, FUGENE®, or polyethylenimine (PEI), for gene delivery to cancerous fibroblasts or human primary fibroblasts. The presently disclosed materials may be useful for cancer therapeutics and regenerative medicine.



(84) Designated States (unless otherwise indicated, for every Published: 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, SE, SI, SK, SM, TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).

- 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))
- (88) Date of publication of the international search report:

31 March 2011

International application No. PCT/US2010/035127

A. CLASSIFICATION OF SUBJECT MATTER

C07C 225/04(2006.01)i, C08G 61/12(2006.01)i, A61K 31/7105(2006.01)i, A61K 47/34(2006.01)i, A61P 35/00(2006.01)i

According to International Patent Classification (IPC) or to both national classification and IPC

B. FIELDS SEARCHED

Minimum documentation searched (classification system followed by classification symbols) C07C 225/04

Documentation searched other than minimum documentation to the extent that such documents are included in the fields searched Korean utility models and applications for utility models

Japanese utility models and applications for utility models

Electronic data base consulted during the international search (name of data base and, where practicable, search terms used) eKOMPASS(KIPO internal) & GOOGLE SCHOLAR

C. DOCUMENTS CONSIDERED TO BE RELEVANT

Category*	Citation of document, with indication, where appropriate, of the relevant passages	Relevant to claim No.
РХ	SUNSHINE, J. et al., Small-Molecule End-Groups of Linear Polymer Determine Cell-type Gene-Delivery Efficacy, Advanced Materials (Weinheim, Germany) (December 2009), 21(48), pp. 4947-4951, ISSN: 0935-9648 See Figures 1-3 and Experimental	1-21,23,24,30-34 ,53-55
X	LEE, JS. et al., Gold, Poly(beta-amino ester) Nanoparticles for Small Interfering RNA Delivery, Nano Letters (07 May 2009), 9(6), pp. 2402-2406, ISSN: 1530-6984	1-21,23,24,30-34 ,53-55
A	See the abstract and Figures 1-5	22,25-29
X	GREEN, J. J. et al., Combinatorial modification of degradable polymers enables transfection of human cells comparable to adenovirus, Advanced Materials (Weinheim, Germany) (2007), 19(19), pp. 2836-2842, ISSN: 0935-9648	1-21,23,24,32-34 ,53-55
A	See Figures 1-4 and Experimental	22,25-31
X	ZUGATES, G. T. et al., Gene Delivery Properties of End-Modified Poly(beta-amino ester), Bioconjugate Chemistry (2007), 18(6), pp. 1887-1896, ISSN: 1043-1802 See the abstract, Introduction, Experimental Section and Figures 1-4	1-21,23,24,32-34 ,53-55

\boxtimes	Further	documents	are	listed	in	the	continuation	of Box	C.
-------------	---------	-----------	-----	--------	----	-----	--------------	--------	----

See patent family annex.

- * Special categories of cited documents:
- "A" document defining the general state of the art which is not considered to be of particular relevance
- "E" earlier application or patent but published on or after the international filing date
- "L" document which may throw doubts on priority claim(s) or which is cited to establish the publication date of citation or other special reason (as specified)
- "O" document referring to an oral disclosure, use, exhibition or other means
- "P" document published prior to the international filing date but later than the priority date claimed
- "T" later document published after the international filing date or priority date and not in conflict with the application but cited to understand the principle or theory underlying the invention
- "X" document of particular relevance; the claimed invention cannot be considered novel or cannot be considered to involve an inventive step when the document is taken alone
- "Y" document of particular relevance; the claimed invention cannot be considered to involve an inventive step when the document is combined with one or more other such documents, such combination being obvious to a person skilled in the art
- "&" document member of the same patent family

Date of mailing of the international search report

Date of the actual completion of the international search

28 JANUARY 2011 (28.01.2011)

28 JANUARY 2011 (28.01.2011)

Authorized officer

Name and mailing address of the ISA/KR



Korean Intellectual Property Office Government Complex-Daejeon, 139 Seonsa-ro, Seogu, Daejeon 302-701, Republic of Korea

Facsimile No. 82-42-472-7140

KANG Young Jin

Telephone No. 82-42-481-8391



INTERNATIONAL SEARCH REPORT

International application No.

PCT/US2010/035127

Box No. II Observations where certain claims were found unsearchable (Continuation of item 2 of first sheet)				
This international search report has not been established in respect of certain claims under Article 17(2)(a) for the following reasons:				
1. Claims Nos.: 36-39, 49-52 because they relate to subject matter not required to be searched by this Authority, namely: Claims 36-39, 49-52 pertain to methods for treatment of the human body by therapy as well as diagnostic methods, and thus relate to a subject matter which this international Searching Authority is not required, under Article 17(2)(a)(i) of the PCT and Rule 39.1(iv) of the Regulations under the PCT, to search.				
Claims Nos.: 42, 46, 57 because they relate to parts of the international application that do not comply with the prescribed requirements to such an extent that no meaningful international search can be carried out, specifically: Claims 42, 46 & 57 are indicated as dependent claims of claims 41, 45 & 56, respectively. However, the claims 41, 45 & 56 do not comply with PCT Rule 6.4(a), and thus they are not searchable, nor are their dependent claims 42, 46 & 57.				
3. Claims Nos.: 35, 40, 41, 43-45, 47, 48, 56 because they are dependent claims and are not drafted in accordance with the second and third sentences of Rule 6.4(a).				
Box No. III Observations where unity of invention is lacking (Continuation of item 3 of first sheet)				
This International Searching Authority found multiple inventions in this international application, as follows:				
1. As all required additional search fees were timely paid by the applicant, this international search report covers all searchable claims.				
2. As all searchable claims could be searched without effort justifying an additional fee, this Authority did not invite payment of any additional fee.				
3. As only some of the required additional search fees were timely paid by the applicant, this international search report covers only those claims for which fees were paid, specifically claims Nos.:				
4. No required additional search fees were timely paid by the applicant. Consequently, this international search report is restricted to the invention first mentioned in the claims; it is covered by claims Nos.:				
Remark on Protest The additional search fees were accompanied by the applicant's protest and, where applicable, the payment of a protest fee. The additional search fees were accompanied by the applicant's protest but the applicable protest fee was not paid within the time limit specified in the invitation. No protest accompanied the payment of additional search fees				

INTERNATIONAL SEARCH REPORT

International application No.

PCT/US2010/035127

C (Continuation). DOCUMENTS CONSIDERED TO BE RELEVANT					
Category*	Citation of document, with indication, where appropriate, of the relevant passages	Relevant to claim No.			
A		22,25-31			
		L			

INTERNATIONAL SEARCH REPORT

Information on patent family members

International application No.

PCT/US2010/035127

Patent document cited in search report	Publication date	Patent family member(s)	Publication date
None			