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

(19) World Intellectual Property Organization
International Bureau

(43) International Publication Date
25 August 2016 (25.08.2016)

WIPO | PCT

(10) International Publication Number
WO 2016/134375 A1

(51) International Patent Classification:
A01N 43/04 (2006.01) *A61K 48/00* (2006.01)
A01N 63/00 (2006.01) *C12N 15/63* (2006.01)
A01N 65/00 (2009.01) *C12N 15/85* (2006.01)

(21) International Application Number:
PCT/US2016/018970

(22) International Filing Date:
22 February 2016 (22.02.2016)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
62/118,934 20 February 2015 (20.02.2015) US

(71) Applicants: **UNIVERSITY OF IOWA RESEARCH FOUNDATION** [US/US]; 112 N. Capitol Street, 6 Gilmore Hall, Iowa City, Iowa 52242-5500 (US). **FONDAZIONE TELETHON** [IT/IT]; Via Varese 16/B, 00185 Rome (IT).

(72) Inventors; and
(71) Applicants : **DAVIDSON, Beverly, L.** [US/US]; 112 N. Capitol Street, 6 Gilmore Hall, Iowa City, Iowa 52242 (US). **CHEN, Yong Hong** [CN/US]; 112 N. Capitol Street, 6 Gilmore Hall, Iowa City, Iowa 52242-5500 (US). **AURICCHIO, Alberto** [IT/IT]; Via Pietro Castellino, 111, 80131 Naples (IT).

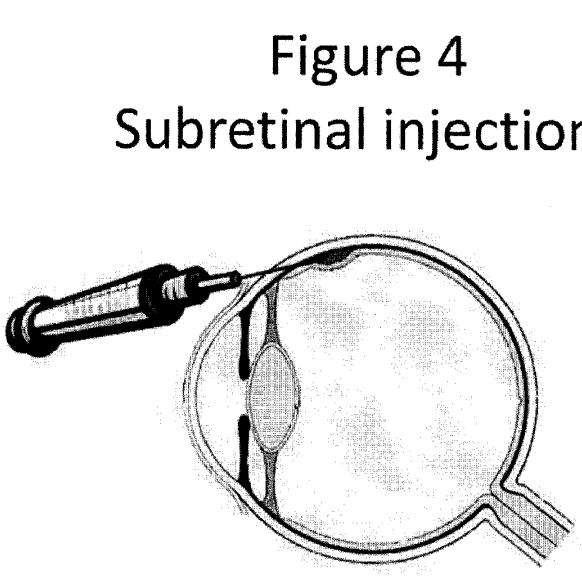
(74) Agents: **VIKSNINS, Ann S.** et al.; Viksnins Harris & Padys PLLP, 7851 Metro Parkway, Suite 325, Bloomington, Minnesota 55425 (US).

(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, DK, DM, DO, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, GT, HN, HR, HU, ID, IL, IN, IR, IS, JP, KE, KG, KN, KP, KR, 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, 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, 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,

[Continued on next page]

(54) Title: METHODS AND COMPOSITIONS FOR TREATING GENETIC EYE DISEASES



(57) Abstract: The present disclosure provides targeting peptides and vectors containing a sequence that encodes targeting peptides that deliver agents, to the eye. The present inventors have discovered peptides that function to target agents, such as viral vectors, to ocular cells. The present disclosure describes a method to utilize these novel peptides to direct, for example, viral capsids to the cell type of interest. In this instance, ocular cells (such as retinal cells) are targeted by the identified peptides. Vectors harboring capsid proteins modified to include such peptides can be used to provide therapeutic agents to the eye.

WO 2016/134375 A1

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:

— *of inventorship (Rule 4.17(iv))*

Published:

— *with international search report (Art. 21(3))*

— *with information concerning incorporation by reference of missing parts and/or elements (Rule 20.6)*