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

(19) World Intellectual Property Organization
International Bureau

(43) International Publication Date
22 August 2013 (22.08.2013)

WIPO | PCT

(10) International Publication Number
WO 2013/123503 A1

(51) International Patent Classification:
C12N 15/00 (2006.01) *A01N 63/00* (2006.01)

(21) International Application Number:
PCT/US2013/026695

(22) International Filing Date:
19 February 2013 (19.02.2013)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
61/600,415 17 February 2012 (17.02.2012) US

(71) Applicant: **THE CHILDREN'S HOSPITAL OF PHILADELPHIA** [US/US]; Colket Translational Reserach Building, 3501 Civic Center Boulevard, Suite 2200-10, Philadelphia, PA 19401 (US).

(72) Inventors: **HIGH, Katherine, A.**; 201 Greenway Lane, Merion Station, PA 19066 (US). **MINGOZZI, Federico**; 326 East Allens Lane, Philadelphia, PA 19119 (US). **SUN, Junwei**; 725 Pemberton Street, Rear 4, Philadelphia, PA 19147 (US). **JOHNSON, Philip**; 206 Indian Creek Road, Wynnewood, PA 19096 (US).

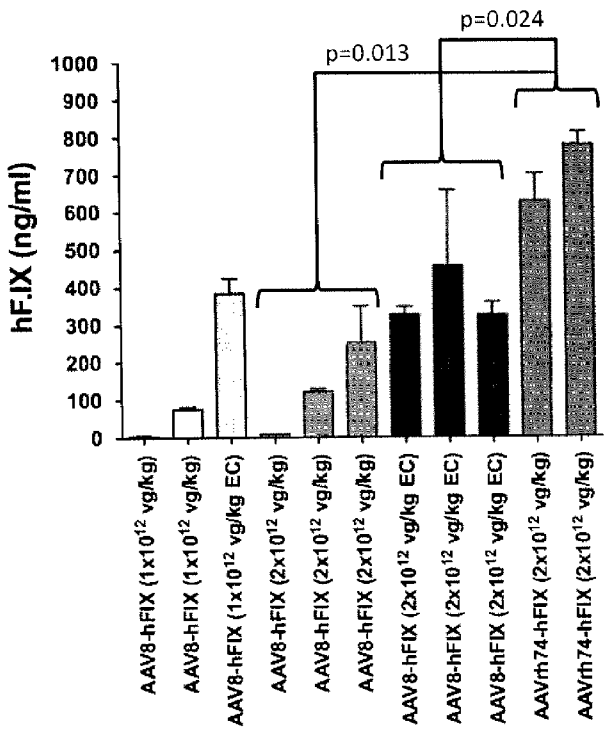
(74) Agent: **BEDGOOD, Robert, M.**; Pillsbury Winthrop Shaw Pittman LLP, P.O. Box 10500, Mclean, VA 22101 (US).

(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, 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, PA, PE, PG, PH, PL, PT, QA, RO, RS, RU, RW, 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, 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, ML, MR, NE, SN, TD, TG).

[Continued on next page]

(54) Title: AAV VECTOR COMPOSITIONS AND METHODS FOR GENE TRANSFER TO CELLS, ORGANS AND TISSUES



(57) Abstract: The invention relates to adeno-associated virus (AAV) serotype AAV-Rh74 and related AAV vectors, and AAV-Rh74 and related AAV vector mediated gene transfer methods and uses. In particular, AAV-Rh74 targets polynucleotides to cells, tissues or organs for expression (transcription) of genes encoding therapeutic proteins and peptides, and polynucleotides that function as or are transcribed into inhibitory nucleic acid sequences.

WO 2013/123503 A1

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))*