



## (51) International Patent Classification:

A01N 63/00 (2006.01) C12N 15/86 (2006.01)  
C12N 15/00 (2006.01)

## (21) International Application Number:

PCT/US2014/038209

## (22) International Filing Date:

15 May 2014 (15.05.2014)

## (25) Filing Language:

English

## (26) Publication Language:

English

## (30) Priority Data:

61/823,757 15 May 2013 (15.05.2013) US

(71) Applicant: REGENTS OF THE UNIVERSITY OF MINNESOTA [US/US]; Suite 600 McNamara Alumni Ctr., 200 Oak Street SE, Minneapolis, Minnesota 55455 (US).

(72) Inventors: MCIVOR, Scott R.; 3745 Glenhurst Avenue South, St. Louis Park, Minnesota 55416 (US). BELUR, Lalitha R.; 1415 Hartford Avenue, St. Paul, Minnesota 55116 (US). LOW, Walter; 19845 Sweetwater Curve, Shorewood, Minnesota 55331 (US). FAIRBANKS, Carolyn; 1874 Grand Avenue, St. Paul, Minnesota 55105 (US).

(74) Agents: PERDOK, Monique M., Reg. No. 42,989 et al.; Schwegman, Lundberg & Woessner, P.A. P.O. Box 2938, Minneapolis, Minnesota 55402 (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, 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, 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, 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, KM, 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))

(54) Title: ADENO-ASSOCIATED VIRUS MEDIATED GENE TRANSFER TO THE CENTRAL NERVOUS SYSTEM

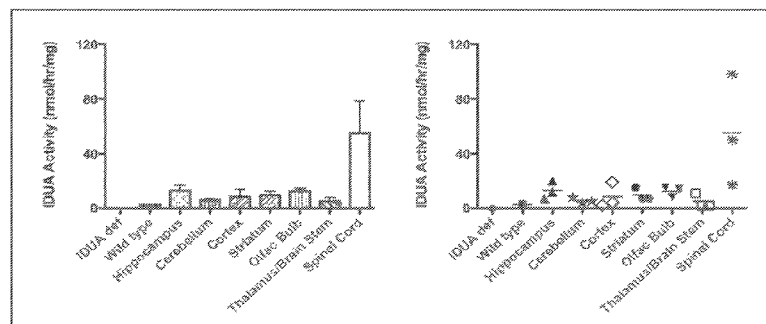


Figure 15

(57) Abstract: A method to prevent, inhibit or treat one or more symptoms associated with a disease of the central nervous system by intrathecally, intracerebroventricularly or endovascularly administering a rAAV encoding a gene product associated with the disease, e.g., a mammal in which the gene product is absent or present at a reduced level relative to a mammal without the disease.