

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
17 August 2023 (17.08.2023)



(10) International Publication Number
WO 2023/154763 A2

- (51) **International Patent Classification:**
C12N 15/86 (2006.01) *A61K 48/00* (2006.01)
- (21) **International Application Number:**
PCT/US2023/062231
- (22) **International Filing Date:**
08 February 2023 (08.02.2023)
- (25) **Filing Language:** English
- (26) **Publication Language:** English
- (30) **Priority Data:**
63/307,963 08 February 2022 (08.02.2022) US

(71) **Applicant:** SPACECRAFT SEVEN, LLC [US/US]; 9 Cedar Brook Drive, Cranbury, New Jersey 08512 (US).

(72) **Inventors:** HERZOG, Christopher Dean; c/o Spacecraft Seven, LLC, 9 Cedar Brook Drive, Cranbury, New Jersey 08512 (US). SACRAMENTO, Chester Bittencort; c/o Spacecraft Seven, LLC, 9 Cedar Brook Drive, Cranbury, New Jersey 08512 (US). PRABHAKAR, Raj; c/o Spacecraft Seven, LLC, 9 Cedar Brook Drive, Cranbury, New Jersey 08512 (US).

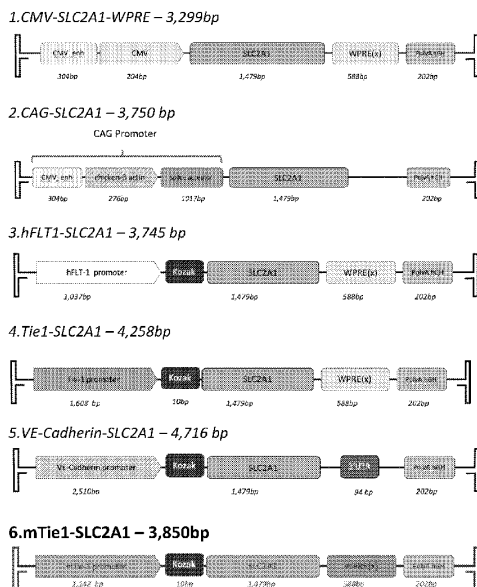
(74) **Agent:** LAHERTY, Carol et al.; Cooley LLP, 1299 Pennsylvania Ave., NW, Suite 700, Washington, District of Columbia 20004 (US).

(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, CV, CZ, DE, DJ, DK, DM, DO, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, GT, HN, HR, HU, ID, IL, IN, IQ, IR, IS, IT, JM, JO, JP, KE, KG, KH, KN, KP, KR, KW, KZ, LA, LC, LK, LR, LS, LU, LY, MA, MD, 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, ST, SV, SY, TH, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, WS, ZA, ZM, ZW.

(84) **Designated States** (*unless otherwise indicated, for every kind of regional protection available*): ARIPO (BW, CV, 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, ME, 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).

(54) **Title:** ADENO-ASSOCIATED VIRAL VECTOR FOR GLUT1 EXPRESSION AND USES THEREOF

FIG. 1



(57) **Abstract:** Provided herein is a gene therapy for GLUT1 Deficiency Syndrome and related disorders using a recombinant adeno-associated virus (rAAV) virion as a vector to express an GLUT1 protein or functional variant thereof. The capsid may be an AAV-BR1 capsid or a functional variant thereof. Other promoters or capsids may be used. The rAAV virion may use an endothelial-specific promoter, e.g., a FLT-1 promoter. Further provided are methods of treatment, such as by intracerebrally and/or intravenously of the rAAV virion, and other compositions and methods.

[Continued on next page]

Published:

- *without international search report and to be republished
upon receipt of that report (Rule 48.2(g))*
- *with sequence listing part of description (Rule 5.2(a))*