

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

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
27 April 2023 (27.04.2023)



(10) International Publication Number
WO 2023/069967 A3

(51) International Patent Classification:

C12N 15/52 (2006.01) *A61P 25/00* (2006.01)
A61K 38/45 (2006.01) *C12N 15/62* (2006.01)
A61K 48/00 (2006.01)

(21) International Application Number:

PCT/US2022/078327

(22) International Filing Date:

18 October 2022 (18.10.2022)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

63/256,827 18 October 2021 (18.10.2021) US

(71) Applicant: **THE TRUSTEES OF THE UNIVERSITY OF PENNSYLVANIA** [US/US]; Penn Center For Innovation, 3600 Civic Center Blvd., 9th Floor, Philadelphia, PA 19104 (US).

(72) Inventors: **WILSON, James, M.**; 1813 Delancey Street, Philadelphia, PA 19103 (US). **SCHMID, Ralf**; 415 Strathmore Road, Havertown, PA 19083 (US).

(74) Agent: **KODROFF, Cathy, A.** et al.; Howson & Howson LLP, 325 Sentry Parkway East, Five Sentry East, Suite 160, Blue Bell, PA 19422 (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, SC, 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).

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))
- with sequence listing part of description (Rule 5.2(a))

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

02 November 2023 (02.11.2023)

(15) Information about Correction:

see Notice of 24 August 2023 (24.08.2023)

(54) Title: COMPOSITIONS USEFUL IN TREATMENT OF CDKL5 DEFICIENCY DISORDER (CDD)

FIG 1A



(57) Abstract: Provided is a recombinant adeno-associated virus (rAAV) having an AAV capsid and a vector genome which comprises a nucleic acid sequence encoding a functional CDKL5 (hCDKL5). Also provided are a production system useful for producing the rAAV, a pharmaceutical composition comprising the rAAV, and a method of treating a subject having CDD, or ameliorating symptoms of CDD, or delaying progression of CDD via administering an effective amount of the rAAV to a subject in need thereof.



WO 2023/069967 A3