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

(19) World Intellectual Property Organization
International Bureau

(43) International Publication Date
24 February 2022 (24.02.2022)

WIPO | PCT

(10) International Publication Number
WO 2022/040432 A1

(51) International Patent Classification:
C12N 7/00 (2006.01)

(21) International Application Number:
PCT/US2021/046699

(22) International Filing Date:
19 August 2021 (19.08.2021)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
63/067,668 19 August 2020 (19.08.2020) US

(71) Applicant: **STRIDEBIO, INC.** [US/US]; P.O. Box 110321, Durham, North Carolina 27709 (US).

(72) Inventor: **O'BANION, Colin**; 3403 Pickett Road, Durham, North Carolina 27705 (US).

(74) Agent: **TUSCAN, Michael S.** et al.; Cooley LLP, 1299 Pennsylvania Ave., 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, CZ, DE, DJ, DK, DM, DO, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, GT, HN, HR, HU, ID, IL, IN, IR, IS, IT, JO, JP, KE, KG, KH, KN, KP, KR, KW, 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, 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, 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, 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:

— *as to applicant's entitlement to apply for and be granted a patent (Rule 4.17(ii))*

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))*

(54) Title: ADENO-ASSOCIATED VIRUS VECTORS FOR TREATMENT OF RETT SYNDROME

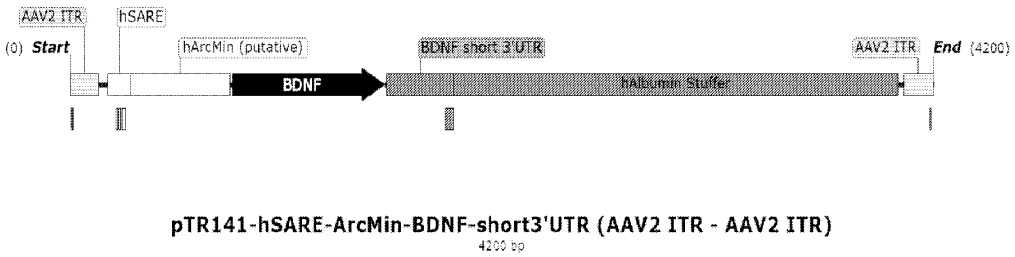

FIG. 1

(57) Abstract: The disclosure provides nucleic acids (comprising AAV expression cassettes), AAV vectors, and compositions for use in methods for treating and/or delaying the onset of diseases associated with mutations in the *mecp2* gene, such as Rett Syndrome. Also, provided herein are methods for treating and/or delaying the onset of brain-derived neurotrophic factor (BDNF)-associated diseases.

WO 2022/040432 A1